BIOMARIN PHARMACEUTICAL INC
Form 10-K
February 26, 2013
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

(Mark One)

x **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the fiscal year ended December 31, 2012

Or

¨ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from to .

Commission file number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware	**68-0397820**
(State of other jurisdiction of	**(I.R.S. Employer**

incorporation or organization)	Identification No.)
770 Lindaro Street	
San Rafael, California	**94901**
(Address of principal executive offices)	**(Zip Code)**

Registrant s telephone number, including area code: (415) 506-6700

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Name of Each Exchange on Which Registered
Common Stock, $.001 par value	The NASDAQ Global Select Market

Securities registered under Section 12(g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes x No ¨

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ¨ No x

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes x No ¨

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No ¨

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant s knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ¨

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of large accelerated filer accelerated filer and smaller reporting company in Rule 12b-2 of the Exchange Act.

Large accelerated filer	x	Accelerated filer	¨
Non-accelerated filer	¨ (Do not check if a smaller reporting company)	Smaller reporting company	¨

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act.) Yes ¨ No x

Indicate the number of shares outstanding of each of the issuer s classes of common stock, as of the latest practicable date: 126,101,610 shares common stock, par value $0.001, outstanding as of February 15, 2013. The aggregate market value of the voting and non-voting stock held by non-affiliates of the registrant as of June 30, 2012 was $2,740.8 million.

The documents incorporated by reference are as follows:

Portions of the Registrant s Proxy Statement for our annual meeting of stockholders to be held May 15, 2013, are incorporated by reference into Part III.

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BIOMARIN PHARMACEUTICAL INC.

2012 FORM 10-K ANNUAL REPORT

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Vimizim is our trademark. BioMarin®, Naglazyme®, Kuvan® and Firdapse® are our registered trademarks. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

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Part I

FORWARD LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements as defined under securities laws. Many of these statements can be identified by the use of terminology such as believes, expects, anticipates, plans, may, will, projects, continues, estimates, pote and similar expressions. These forward-looking statements may be found in *Risk Factors*, *Business*, and other sections of this Annual Report on Form 10-K. Our actual results or experience could differ significantly from the forward-looking statements. Factors that could cause or contribute to these differences include those discussed in *Risk Factors*, as well as those discussed elsewhere in this Annual Report on Form 10-K. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may issue in the future. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Annual Report on Form 10-K to reflect later events or circumstances or to reflect the occurrence of unanticipated events.

The following discussion of our financial condition and results of operations should be read in conjunction with our Consolidated Financial Statements and the notes thereto appearing elsewhere in this Annual Report on Form 10-K. In addition to the other information in this Annual Report on Form 10-K, investors should carefully consider the following discussion and the information under *Risk Factors* when evaluating us and our business.

Item 1. Business

Overview

BioMarin Pharmaceutical Inc. (BioMarin, we, us or our) develops and commercializes innovative pharmaceuticals for serious diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. Our product portfolio is comprised of four approved products and multiple investigational product candidates. Approved products include Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Naglazyme received marketing approval in the United States (U.S.) in May 2005, in the European Union (EU) in January 2006 and subsequently in other countries. Kuvan was granted marketing approval in the U.S. and EU in December 2007 and December 2008, respectively. In December 2009, the European Medicines Agency (EMEA) granted marketing approval for Firdapse, which was launched in the EU in April 2010. Aldurazyme, which was developed in collaboration with Genzyme Corporation (Genzyme), was approved in 2003 for marketing in the U.S., EU and subsequently other countries. Net product revenues during 2012 for our approved products, Naglazyme, Kuvan, Firdapse and Aldurazyme were $257.0 million, $143.1 million, $14.2 million and $82.2 million, respectively.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: Vimizim (formerly referred to as GALNS), an enzyme replacement therapy for the treatment of Mucopolysaccharidosis Type IV or Morquio Syndrome Type A, or MPS IV A, PEG-PAL, an enzyme substitution therapy for the treatment of phenylketonuria or PKU, BMN-701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder, BMN-673, an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers and BMN-111, a peptide therapeutic for the treatment of achondroplasia.

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We are conducting or planning to conduct preclinical development of several other enzyme product candidates for genetic and other metabolic diseases, including BMN-190 for the treatment of late infantile neuronal ceroid lipofuscinosis, or LINCL, a form of Batten disease. We expect to initiate a Phase 1 clinical trial of BMN-190 in the first half of 2013.

A summary of our various commercial products and major development programs, including key metrics as of December 31, 2012, is provided below:

Program	Indication	Orphan Drug Designation	Stage	2012 Total Net Product Revenues (in millions)	2012 Research & Development Expense (in millions)
Naglazyme	MPS VI (1)	Yes	Approved	$ 257.0	$ 12.4
Aldurazyme (2)	MPS I (3)	Yes	Approved	$ 82.2	$ 1.3
Kuvan	PKU (4)	Yes	Approved	$ 143.1	$ 14.1
Firdapse (5)	LEMS (6)	Yes	Approved in the EU only	$ 14.2	$ 5.4
Vimizim for MPS IV A	MPS IVA(7)	Yes	Clinical Phase 3	N/A	$ 97.0
PEG-PAL	PKU	Yes	Clinical Phase 2	N/A	$ 26.7
BMN-701 for Pompe disease	POMPE (8)	Yes	Clinical Phase 1/2	N/A	$ 31.6
BMN-673, PARP inhibitor for the treatment of patients with cancer	Not yet determined	Not yet determined	Clinical Phase 1/2	N/A	$ 9.7
BMN-673, PARP inhibitor for the treatment of patients with hematological malignancies	Not yet determined	Not yet determined	Clinical Phase 1/2	N/A	$ 1.7
BMN-111, peptide therapeutic for the treatment of Achondroplasia	Achondroplasia	Yes	Clinical Phase 1	N/A	$ 12.1

(1) Mucopolysaccharidosis VI, or MPS VI
(2) The Aldurazyme total product revenue noted above is the total product revenue recognized by us in accordance with the terms of our agreement with Genzyme Corporation. See *Commercial Products Aldurazyme* below for further discussion.
(3) Mucopolysaccharidosis I, or MPS I
(4) Phenylketonuria, or PKU
(5) Marketing approval from the EMEA for Firdapse was granted in December 2009. We launched Firdapse in the EU in April 2010.
(6) Lambert Eaton Myasthenic Syndrome, or LEMS
(7) Morquio A Syndrome, or MPS IVA
(8) Pompe disease, a glycogen storage disorder

Commercial Products

Naglazyme

Naglazyme is a recombinant form of N-acetylgalactosamine 4-sulfatase (arylsulfatase B) indicated for patients with mucopolysaccharidosis VI, or MPS VI. MPS VI is a debilitating life-threatening genetic disease for which no other drug treatment currently exists and is caused by the deficiency of arylsulfatase B, an enzyme normally required for the breakdown of certain complex carbohydrates known as glycosaminoglycans, or GAGs. Patients with MPS VI typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of carbohydrate residues in tissues in the body. These symptoms include: inhibited growth, spinal cord compression, enlarged liver and spleen, joint deformities and reduced range of motion, skeletal deformities, impaired cardiovascular function, upper airway obstruction, reduced pulmonary function, frequent ear and lung infections, impaired hearing and vision, sleep apnea, malaise and reduced endurance.

Naglazyme was granted marketing approval in the U.S. in May 2005 and in the EU in January 2006. We market Naglazyme in the U.S., EU, Canada, Latin America, Turkey, and Russia using our own sales force and commercial organization. Additionally, we use local distributors in several other regions to help us pursue registration and/or market Naglazyme on a named patient basis. Naglazyme net product sales for 2012 totaled $257.0 million, as compared to $224.9 million for 2011. Naglazyme net product sales for 2010 were $192.7 million.

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Kuvan

Kuvan is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for phenylalanine hydroxylase, or PAH, indicated for patients with PKU. Kuvan is the first drug for the treatment of PKU, which is an inherited metabolic disease that affects at least 50,000 diagnosed patients under the age of 40 in the developed world. We believe that approximately 30 to 50% of those with PKU could benefit from treatment with Kuvan. PKU is caused by a deficiency of activity of an enzyme, PAH, which is required for the metabolism of phenylalanine, or Phe. Phe is an essential amino acid found in all protein-containing foods. Without sufficient quantity or activity of PAH, Phe accumulates to abnormally high levels in the blood, resulting in a variety of serious neurological complications, including severe mental retardation and brain damage, mental illness, seizures and other cognitive problems.

Kuvan was granted marketing approval for the treatment of PKU in the U.S. in December 2007. We market Kuvan in the U.S. and Canada using our own sales force and commercial organization. Kuvan has been granted orphan drug status in the U.S., which confers seven years of market exclusivity in the U.S. for the treatment of PKU, expiring in 2014. We expect that our patents will provide market exclusivity beyond the expiration of orphan status. Kuvan net product sales for 2012 were $143.1 million, as compared to $116.8 million for 2011. Kuvan net product sales for 2010 were $99.4 million.

In May 2005, we entered into an agreement with Merck Serono S.A. (Merck Serono) for the further development and commercialization of Kuvan and any other product containing 6R-BH4, and PEG-PAL for PKU. Through the agreement, as amended in 2007, Merck Serono acquired exclusive rights to market these products in all territories outside the U.S., Canada and Japan, and we retained exclusive rights to market these products in the U.S. and to market Kuvan in Canada. Merck Serono markets Kuvan in the EU and several other countries outside the U.S., Canada and Japan. Under the agreement with Merck Serono, we are entitled to receive royalties, on a country-by-country basis, until the later of the expiration of patent right licensed to Merck Serono or ten years after the first commercial sale of the licensed product in such country. Over the next several years, we expect a royalty of approximately four percent on net sales of Kuvan by Merck Serono. We also sell Kuvan to Merck Serono at or near cost, and Merck Serono resells the product to end-users outside the U.S., Canada and Japan. The royalty earned from Kuvan product sold by Merck Serono in the EU is included as a component of net product revenues in the period earned. In 2012, we earned $1.9 million in net royalties on net sales of $46.8 million of Kuvan by Merck Serono, compared to 2011 when we earned $1.6 million in net royalties on net sales of $40.4 million. In 2010, we earned $0.9 million in net royalties on net sales of $23.7 million. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of $2.0 million in 2012, $0.5 million in 2011 and $0.7 million in 2010.

On February 19, 2013, we announced results from our PKU-016 ASCEND study, a randomized controlled trial evaluating neuropsychiatric outcomes in PKU patients treated with Kuvan. The study evaluated medically important symptoms similar to attention deficit hyperactivity disorder (ADHD) in PKU patients whose blood levels of Phe are reduced by Kuvan. The primary endpoint of the study was evaluated using an attention deficit hyperactivity rating scale (ADHD-RS), commonly used to evaluate symptoms of inattentiveness and hyperactivity. Kuvan improved the ADHD-RS ($p=0.085$), driven by a statistically significant change in the inattention component of the score ($p=0.036$).

Aldurazyme

Aldurazyme has been approved for marketing in the U.S., EU and other countries for patients with mucopolysaccharidosis I, or MPS I. MPS I is a progressive and debilitating life-threatening genetic disease, for which no other drug treatment currently exists, that is caused by the deficiency of alpha-L-iduronidase, a lysosomal enzyme normally required for the breakdown of GAGs. Patients with MPS I typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of carbohydrate residues in all tissues in the body. These symptoms include: inhibited growth, delayed and

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regressed mental development (in the severe form of the disease), enlarged liver and spleen, joint deformities and reduced range of motion, impaired cardiovascular function, upper airway obstruction, reduced pulmonary function, frequent ear and lung infections, impaired hearing and vision, sleep apnea, malaise and reduced endurance.

We developed Aldurazyme through collaboration with Genzyme. Under our collaboration agreement, we are responsible for manufacturing Aldurazyme and supplying it to Genzyme. Genzyme records sales of Aldurazyme and is required to pay us, on a quarterly basis, a 39.5% to 50% royalty on worldwide net product sales. We recognize a portion of this royalty as product transfer revenue when product is released to Genzyme and all of our obligations have been fulfilled. Genzyme s return rights for Aldurazyme are limited to defective product. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay us if the product is unsold by Genzyme. The amount of product transfer revenue will eventually be deducted from the calculated royalty when the product is sold by Genzyme. Additionally, Genzyme and we are members of a 50/50 limited liability company that: (1) holds the intellectual property relating to Aldurazyme and other collaboration products and licenses all such intellectual property on a royalty-free basis to us and Genzyme to allow us to exercise our rights and perform our obligations under the agreements related to the restructuring, and (2) engages in research and development activities that are mutually selected and funded by Genzyme and us.

Our Aldurazyme net product revenues totaled $82.2 million for 2012 as compared to $82.8 million for 2011 and $71.2 million for 2010. The net product revenues for 2012, 2011 and 2010 include $80.4 million, $74.2 million and $68.0 million, respectively, of royalty revenue on net Aldurazyme sales by Genzyme. Net sales of Aldurazyme by Genzyme totaled $193.1 million for 2012, $185.2 million for 2011 and $166.8 million for 2010. Incremental Aldurazyme net product transfer revenue of $1.8 million, $8.6 million, and $3.2 million for 2012, 2011 and 2010, respectively, reflect incremental shipments of Aldurazyme to Genzyme to meet future product demand. In the future, to the extent that Genzyme Aldurazyme inventory quantities on hand remain consistent, we expect that our total Aldurazyme revenues will approximate the 39.5% to 50% royalties on net product sales by Genzyme.

Firdapse

Firdapse is a form of 3, 4-diaminopyridine (amifampridine phosphate), or 3, 4-DAP for the treatment of LEMS. Firdapse was originally developed by AGEPS, the pharmaceutical unit of the Paris Public Hospital Authority, or AP-HP. Firdapse was granted marketing approval in the EU in December 2009. In addition, Firdapse has been granted orphan drug status in the EU, which confers ten years of market exclusivity in the EU. We launched Firdapse on a country-by-country basis in Europe beginning in April 2010. Firdapse net product revenues in 2012 were $14.2 million, compared to $13.1 million and $6.4 million in 2011 and 2010, respectively. In October 2012, we licensed to Catalyst Pharmaceutical Partners, Inc. the North American rights to develop and market Firdapse. In exchange for the North American rights to Firdapse, we may receive royalties of 7% to 10% on net product sales of Firdapse in North America.

LEMS is a rare autoimmune disease with the primary symptoms of muscle weakness. Muscle weakness in LEMS is caused by autoantibodies to voltage gated calcium channels leading to a reduction in the amount of acetylcholine released from nerve terminals. The prevalence of LEMS is estimated at four to ten per million, or approximately 2,000 to 5,000 patients in the EU and 1,200 to 3,100 patients in the U.S. Approximately 50% of LEMS patients diagnosed have small cell lung cancer. Patients with LEMS typically present with fatigue, muscle pain and stiffness. The weakness is generally more marked in the proximal muscles particularly of the legs and trunk. Other problems include reduced reflexes, drooping of the eyelids, facial weakness and problems with swallowing. Patients often report a dry mouth, impotence, constipation and feelings of light headedness on standing. On occasion these problems can be life threatening when the weakness involves respiratory muscles. A diagnosis of LEMS is generally made on the basis of clinical symptoms, electromyography testing and the presence of auto antibodies against voltage gated calcium channels. Currently approved treatments of LEMS can

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consist of strategies directed at the underlying malignancy, if one is present. Therapy of small cell lung cancer is limited and outcomes are generally poor. Immunosuppressive agents have been tried but success is limited by toxicity and difficulty administering the regimens. A mainstay of therapy has been 3, 4-DAP, but its use in practice has been limited by the drug s availability.

Products in Clinical Development

We are developing Vimizim, an enzyme replacement therapy for the treatment of MPS IV A, a lysosomal storage disorder. In November 2012, we announced the top-line results of a pivotal Phase 3 clinical trial for Vimizim for the treatment of MPS IV A. The top-line results demonstrated that the study met the primary endpoint of change in six-minute walk distance compared with a placebo at 24 weeks in subjects receiving weekly infusions of Vimizim at the dose of two milligrams per kilogram per week. This Phase 3 trial was a randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of Vimizim in patients with MPS IV A. The trial was conducted at 31 centers worldwide including Brazil, Japan, Taiwan, most Western European countries, Canada and the U.S. We enrolled 176 patients in this trial. The trial explored doses of two milligrams per kilogram per week and two milligrams per kilogram every other week for a treatment period of 24 weeks.

In addition, in November 2011, we announced the initiation of a Phase 2 study for Vimizim in patients with MPS IVA who are under five years of age. The primary objective of the Phase 2, open-label, multinational clinical study is to evaluate the safety and tolerability of infusions of Vimizim at a dose of 2.0 milligrams per kilogram per week over a 52-week period in 10 to 15 patients with MPS IVA who are under five years of age. The secondary objectives are to evaluate urinary keratin sulfate levels and growth velocity. This study is ongoing.

PEG-PAL is an investigational enzyme substitution therapy that we are developing as a subcutaneous injection for the treatment of PKU. In preclinical models, PEG-PAL produced a rapid, dose-dependent reduction in blood phenylalanine, or Phe levels, the same endpoint that was used in the Kuvan studies. In June 2009, we announced results from a Phase 1 open-label, single-dose, dose-escalation clinical trial of PEG-PAL for PKU. Significant reductions in blood Phe levels were observed in all patients in the fifth dosing cohort of the Phase 1 trial. In addition, there were no serious immune reactions observed and mild to moderate injection-site reactions were in line with our expectations. In September 2009, we initiated a Phase 2, open-label dose finding clinical trial of PEG-PAL. The primary objective of this clinical trial is to optimize the dose and schedule that produces the most favorable safety profile and Phe reduction. The secondary objectives of the clinical trial are to evaluate the safety and tolerability of multiple dose levels of PEG-PAL, to evaluate the immune response to PEG-PAL, and to evaluate steady-state phamacokinetics in all patients and accumulation of PEG-PAL in a subset of patients enrolled in this clinical trial. Preliminary results from this clinical trial were presented in August 2010 and showed that of the seven patients who received at least one milligram per kilogram per week of PEG-PAL for at least four weeks, six patients have achieved Phe levels below 600 micromoles per liter. Mild to moderate self-limiting injection site reactions are the most commonly reported toxicity. In April 2011 we initiated an extension of the Phase 2 study to find the quickest and safest induction dosing regimen to an efficacious maintenance dose. This study is ongoing. We expect to initiate a Phase 3 clinical trial of PEG-PAL in the second quarter of 2013.

BMN-673 is a PARP inhibitor, a class of molecules that has shown clinical activity against cancers involving defects in DNA repair that we are investigating for the treatment of certain cancers. In January 2011, we announced the initiation of a Phase 1/2 clinical trial for BMN-673 for the treatment of patients with solid tumors. The clinical trial is an open-label study of once daily, orally administered BMN-673 in approximately 85 patients ages 18 and older with advanced or recurrent solid tumors. The primary objective of the study is to establish the maximum tolerated dose of daily oral BMN-673. The secondary objective of the study is to establish the safety, pharmacokinetic profile and recommended Phase 2 dose. The study, to date, has established a preliminary dose that is generally well-tolerated and reaches steady state with repeated daily doses, and we are currently expanding the number of patients treated at that dose. The expansion phase of the study will focus on cancers characterized by BRCA mutations, Ewing s sarcoma and small cell lung cancer.

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In July 2011, we initiated a Phase 1/2 clinical trial for BMN-673 for the treatment of patients with advanced hematological malignancies. This clinical trial is a two-arm, open-label dose escalation study to determine the maximum tolerated dose and to assess the safety, pharmacokinetics, pharmacodynamics and preliminary efficacy of once daily, orally administered BMN-673 in patients with acute myeloid leukemia, myelodsplastic syndrome, chronic lymphocytic leukemia or mantle cell lymphoma. This study will enroll approximately 80 patients. Currently, the study is in a dose-escalation phase.

BMN-701 is a novel fusion of insulin-like growth factor 2 and alpha glucosidase (IGF2-GAA) in development for Pompe disease. We acquired the BMN-701 program in August 2010 in connection with the acquisition of ZyStor Therapeutics, Inc. (ZyStor). In January 2011, we announced the initiation of a Phase 1/2 clinical trial for BMN-701. This clinical trial is an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN-701 administered as an intravenous infusion every two weeks at doses of 20 milligrams per kilogram. We have completed enrollment of this study with 22 patients between the ages of 13 and 65 years old with late-onset Pompe disease for a treatment period of 24 weeks. The primary objectives of this study are to evaluate the safety and tolerability of BMN-701 as well as determine the antibody response to BMN-701. The secondary objectives of the study are to determine the single and multi-dose pharmacokinetics of BMN-701 and determine mobility and functional exercise capacity in patients receiving BMN-701. Pompe disease is a lysosomal storage disorder caused by a deficiency in GAA, which prevents cells from adequately degrading glycogen. This results in the storage of glycogen in lysosomes, particularly those in muscle cells, thereby damaging those cells and causing progressive muscle weakness which in turn can result in death due to pulmonary or cardiac insufficiency. We expect to report top-line results from this study in the first quarter of 2013.

BMN-111 is a peptide therapeutic in development for the treatment of achondroplasia. In September 2012, we announced the results of a Phase 1 clinical trial for BMN-111. The primary objective of the Phase 1 clinical trial was to assess the safety and tolerability of single and multiple doses of BMN-111 in normal healthy adult volunteers up to the maximum tolerated dose. BMN-111 was generally well-tolerated over the range of single and repeat doses studied. Pharmacokinetic data indicated that the dose levels studied resulted in exposure levels that are expected to stimulate growth based on non-clinical findings. We expect to start the Phase 2 study in pediatric patients in mid-2013.

Manufacturing

We manufacture Naglazyme, Aldurazyme, Vimizim, PEG-PAL and BMN-111 in our approved Good Manufacturing Practices (GMP) production facilities located in Novato, California. Vialing and packaging are performed by contract manufacturers. We believe that we have ample operating capacity to support the commercial demand of both Naglazyme and Aldurazyme through at least the next five years as well as the clinical requirements and initial launch of Vimizim, if approved.

In August 2011, we acquired a bulk biologics manufacturing plant located in Shanbally, County of Cork, Ireland. This 142,000-square-foot facility which was completed and validated in 2009 was approved by the Irish Medicines Board in 2010. We are not currently manufacturing any products in this facility. We currently intend to manufacture Vimizim in this facility. However, before we can manufacture any product in this facility, including Vimizim, substantial modifications to the facility will be required and we will need to requalify and validate certain systems in the facility. The addition of the Shanbally facility will increase our operating capacity to support the commercial demand of Vimizim, if approved.

Our Novato, California facilities have been licensed by the Food and Drug Administration (FDA), the European Commission (EC) and health agencies in other countries for the commercial production of Aldurazyme and Naglazyme. All of our facilities and those of any third-party manufacturers will be subject to periodic inspections confirming compliance with applicable law and must be GMP certified before we can manufacture our drugs for commercial sales.

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Kuvan is manufactured on a contract basis by a third-party. There are two approved manufacturers of the active pharmaceutical ingredient, or API, for Kuvan. Firdapse, BMN-701 and BMN-673 are each manufactured on a contract basis by a third-party. There is one approved manufacturer of the API for Firdapse.

In general, we expect to continue to contract with outside service providers for certain manufacturing services, including final product vialing and packaging operations for our recombinant enzymes and API production and tableting for Kuvan and Firdapse. Third-party manufacturers facilities are subject to periodic inspections to confirm compliance with applicable law and must be GMP certified. We believe that our current agreements with third-party manufacturers and suppliers provide for ample operating capacity to support the anticipated commercial demand for Kuvan and Firdapse. In certain instances, there is only one approved contract manufacturer for certain aspects of the manufacturing process. In such cases, we attempt to prevent disruption of supplies through supply agreements, maintaining safety stock and other appropriate strategies. Although we have never experienced a disruption in supply from our contract manufacturers, we cannot provide assurance that we will not experience a disruption in the future.

Raw Materials

Raw materials and supplies required for the production of our products and product candidates are available, in some instances from one supplier, and in other instances, from multiple suppliers. In those cases where raw materials are only available through one supplier, such supplier may be either a sole source (the only recognized supply source available to us) or a single source (the only approved supply source for us among other sources). We have adopted policies to attempt, to the extent feasible, to minimize our raw material supply risks, including maintenance of greater levels of raw materials inventory and implementation of multiple raw materials sourcing strategies, especially for critical raw materials. Although to date we have not experienced any significant delays in obtaining any raw materials from our suppliers, we cannot provide assurance that we will not face shortages from one or more of them in the future.

Sales and Marketing

We have established a commercial organization, including a small salesforce to support our product lines directly in the U.S., Europe, South America and certain other significant markets. For other selected markets, we have signed agreements with other companies to act as distributors of Naglazyme. Most of these agreements generally grant the distributor the right to market the product in the territory and the obligation to secure all necessary regulatory approvals for commercial or named patient sales. Additional markets are being assessed at this time and additional agreements may be signed in the future. We believe that the size of our sales force is appropriate to effectively reach our target audience in markets where Naglazyme, Kuvan and Firdapse are directly marketed. We utilize third-party logistics companies to store and distribute our products.

Genzyme has the exclusive right to distribute, market and sell Aldurazyme globally and is required to purchase its requirements exclusively from us.

Customers

Our Naglazyme, Kuvan and Firdapse customers include a limited number of specialty pharmacies and end-users, such as hospitals and foreign government agencies, which act as retailers. We also sell Naglazyme to our authorized European distributors and to certain larger pharmaceutical wholesalers, which act as intermediaries between us and end-users and generally do not stock significant quantities of Naglazyme. During 2012, 43% of our net Naglazyme, Kuvan and Firdapse product revenues were generated by three customers. Genzyme is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties.

Despite the significant concentration of customers, the demand for Naglazyme, Kuvan and Firdapse is driven primarily by patient therapy requirements and we are not dependent upon any individual distributor with respect to Naglazyme, Kuvan or Firdapse sales. Due to the pricing of Naglazyme, Kuvan and Firdapse and the limited number of patients, the specialty pharmacies and wholesalers generally carry a very limited inventory, resulting in sales of Naglazyme, Kuvan and Firdapse being closely tied to end-user demand. However, in certain countries particularly in Latin America, governments place large periodic orders for Naglazyme. The timing of these orders can create significant quarter to quarter variation in our revenue.

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Competition

The biopharmaceutical industry is rapidly evolving and highly competitive. The following is a summary analysis of known competitive threats for each of our major product programs:

Naglazyme, Aldurazyme and Vimizim

Small companies and academic groups continue to evaluate various approaches to treating MPS VI, MPS I or MPS IVA however, we are not aware of any active competitive program for enzyme replacement therapy for MPS VI, MPS I or MPS IV A that has entered clinical trials.

Bone marrow transplantation has been used to treat severely affected patients, generally under the age of two, with some success. Bone marrow transplantation is associated with high morbidity and mortality rates as well as with problems inherent in the procedure itself, including graft versus host disease, graft rejection and donor availability, which limits its utility and application. There are other developing technologies, including gene therapy, that are potential competitive threats to enzyme replacement therapies. However, we know of no such technology that has entered clinical trials related to MPS VI, MPS I or MPS IV A.

Kuvan and PEG-PAL

There are currently no other approved drugs for the treatment of PKU. PKU is commonly treated with a medical food diet that is highly-restrictive and unpalatable. We perceive medical foods as a complement to Kuvan and PEG-PAL and not a significant competitive threat. Dietary supplements of large neutral amino acids (LNAA), have also been used in the treatment of PKU. This treatment may be a competitive threat to Kuvan and PEG-PAL. However, because LNAA is a dietary supplement, the FDA has not evaluated any claims of efficacy of LNAA. At least one company has filed a drug master file with the FDA for production of the active ingredient in Kuvan. However, we have no knowledge that any company has filed an abbreviated new drug application, or ANDA, for Kuvan or performed the bioequivalence study that would be required for an ANDA. See the ANDA discussion under The Hatch-Waxman Act for additional information.

Firdapse and LEMS

There are no other approved drugs for the treatment of LEMS. Current options rely on intravenous immunoglobulin, plasmapherisis and/or immuno suppressant drugs. In some countries, 3,4 DAP is available, as a base, through various compounding pharmacies, as a special or magistral formulation, or through investigator sponsored studies. Firdapse is the only approved version of 3,4 DAP. One other aminopyridine, 4AP, has been approved in the U.S. by another pharmaceutical company. However, this is for the treatment of fatigue associated with Multiple Sclerosis. The role of 4AP in LEMS is unproven and uncertain.

BMN-673

There are several other PARP inhibitors ahead of BMN-673 in clinical development for the treatment of various solid and hematologic malignancies. None of these PARP inhibitors however, has yet been approved by the FDA or any other regulatory agency.

BMN-701

There are two approved enzyme replacement therapies for Pompe disease in the U.S. and at least two more in preclinical studies. Gene therapy is also being tested in clinical trials and a pharmaceutical company initiated a Phase 2 clinical trial to test its small molecule chaperone as a combination therapy with enzyme replacement therapy.

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BMN-111

There are currently no approved drugs for the treatment of achondroplasia. There are other peptides in early development for achondroplasia, although BMN-111 is the only peptide therapeutic that has entered clinical trials for achondroplasia.

Patents and Proprietary Rights

Our success depends on an intellectual property portfolio that supports our future revenue streams and also erects barriers to our competitors. We are maintaining and building our patent portfolio through: filing new patent applications; prosecuting existing applications and licensing and acquiring new patents and patent applications. Furthermore we seek to protect our ownership of know-how, trade secrets and trademarks through an active program of legal mechanisms including registrations, assignments, confidentiality agreements, material transfer agreements, research collaborations and licenses.

The number of our issued patents now stands at approximately 213, including approximately 62 patents issued by the U.S. Patent and Trademark Office (USPTO). Furthermore, our portfolio of pending patent applications totals approximately 354 applications, including approximately 58 pending U.S. applications.

With respect to Naglazyme, we have 17 issued patents, including three U.S. patents. Claims cover our ultrapure *N* -acetylgalactosamine-4-sulfatase compositions of Naglazyme, methods of treating deficiencies of *N* -acetylgalactosamine-4-sulfatase, including MPS VI, methods of producing and purifying such ultrapure *N* -acetylgalactosamine-4-sulfatase compositions, and methods of detecting lysosomal enzyme-specific antibodies. These patents will expire between 2022 (compositions of matter, methods of use) and 2028 (methods of detecting).

With respect to Kuvan and BH4, we own, co-own or have licensed a number of patents and pending patent applications that relate generally to formulations and forms of our drug substance, methods of use for various indications under development and dosing regimens. We have rights to 31 issued patents including 12 issued U.S. patents with claims to a stable tablet formulation of BH4, methods of treating PKU using a once daily dosing regimen, methods of administration of Kuvan with food, crystalline forms of BH4, and methods of producing BH4. These patents will expire between 2024 and 2029.

We have rights to 33 issued patents, including six U.S. patents, related to Aldurazyme. These patents cover our ultra-pure alpha-L-iduronidase composition of Aldurazyme, methods of treating deficiencies of alpha-L-iduronidase by administering pharmaceutical compositions comprising such ultra-pure alpha-L-iduronidase, a method of purifying such ultra-pure alpha-L-iduronidase and the use of compositions of ultra-pure biologically active fragments of alpha-L-iduronidase. These patents will expire in 2019 and 2020. There are U.S. patents on alpha-L-iduronidase owned and controlled by a third-party. We have examined such issued U.S. patents, the related U.S. and foreign applications and their file histories, the prior art and other information. Corresponding foreign applications were filed in Canada, Europe and Japan. The European application was rejected and abandoned and cannot be re-filed. The Japanese application has also lapsed and cannot be re-filed. Claims in the related Canadian application issued in 2007. We believe that such patents may not survive a challenge to patent validity but that it is unlikely that a court in any country would order us to stop marketing the only life-saving drug that is currently approved for this disease. However, the processes of patent law are uncertain and any patent proceeding is subject to multiple unanticipated outcomes. We believe that it is in the best interest of our joint venture with Genzyme to market Aldurazyme with commercial diligence, in order to provide MPS I patients with the benefits of Aldurazyme. We believe that these patents and patent applications do not affect our ability to market Aldurazyme in Europe.

We have patent protection in the European Patent Organization (EPO) countries for Firdapse for the treatment of LEMS and we have no issued patents in the U.S. for Firdapse for the treatment of LEMS.

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With respect to Vimizim, we own or have licensed a number of patents and pending patent applications that relate generally to compositions of matter, methods of use and methods of production. We have rights to 11 issued patents including five issued U.S. patents with claims to compositions of purified recombinant N-acetylgalactosamine-6-sulfate sulfatase (Vimizim) methods of treating Morquio Syndrome and sulfatase-modifying factor I (SUMF1) polypeptides and nucleic acids used in the manufacture of Vimizim. Issued U.S. patents cover SUMF1 compositions (set to expire in 2019), purified recombinant Vimizim compositions (set to expire in 2029) and methods of treating Morquio Syndrome (set to expire in 2029). We also have issued U.S. and European patents that cover methods of production and are set to expire in 2024.

Government Regulation

We operate in a highly regulated industry, which is subject to significant federal, state, local and foreign regulation. Our present and future business has been, and will continue to be, subject to a variety of laws including the Federal Food, Drug and Cosmetic Act, or FDC Act, the Public Health Service Act, the Medicaid rebate program, the Veterans Health Care Act of 1992, and the Occupational Safety and Health Act, among others.

The FDC Act and other federal and state statutes and regulations govern, among other things, the testing, research, development, manufacture, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, import and export of our products. As a result of these laws and regulations, product development and product approval processes are very expensive and time consuming.

FDA Approval Process

Pharmaceutical product development in the U.S. typically involves preclinical laboratory and animal tests, the submission to the FDA of an investigational new drug application, or IND, which must become effective before clinical testing may commence, and adequate and well-controlled human clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation, as well as animal trials, to assess the characteristics and potential pharmacology and toxicity of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol. Long term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has not objected to the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, good clinical practices, or GCP, as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB s requirements, or may impose other conditions.

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Clinical trials to support new drug applications, or NDAs, or biological product licenses, or BLAs, for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population, to determine the effectiveness of the drug for a particular indication or indications, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites. After completion of the required clinical testing, an NDA or BLA is prepared and submitted to the FDA. FDA approval of the NDA or BLA is required before marketing of the product may begin in the U.S. The NDA or BLA must include the results of all preclinical, clinical and other testing, a compilation of data relating to the product s pharmacology, chemistry, manufacture and controls, proposed labeling and a payment of a significant user fee (currently exceeding $1,958,000), among other things. The manufacturer and/or sponsor under an approved NDA or BLA are also subject to annual product and establishment user fees, currently exceeding $98,000 per product and $526,000 per establishment. These fees are typically increased by the FDA annually.

The FDA has 60 days from its receipt of an NDA or BLA to determine whether the application will be accepted for filing based on the agency s threshold determination that it is sufficiently complete to permit substantive review. The FDA may request additional information rather than accepting an NDA or BLA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs or BLAs. Most such applications for non-priority drug products are reviewed within ten to twelve months. The goal for initial review of most applications for priority review of drugs, that is, drugs that the FDA determines represent a significant improvement over existing therapy, is six months to eight months. The review process may be extended by the FDA for three additional months to consider new information submitted during the review or clarification regarding information already provided in the submission. The FDA may also refer applications for novel products or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with current good manufacturing practices, or cGMPs, is satisfactory and the NDA or BLA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA or BLA, including the manufacturing procedures and facilities, it issues an approval letter, or a complete response letter. A complete response letter outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed, the FDA will re-initiate review. If it is satisfied that the deficiencies have been addressed, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. It is not unusual, however, for the FDA to issue a complete response letter because it believes that the drug is not safe enough or effective enough or because it does not believe that the data submitted are reliable or conclusive.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA or BLA approval, the FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. The

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requirement for REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug s safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs and biologics, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial are then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly-available information to gain knowledge regarding the progress of development programs.

The Hatch-Waxman Act

Upon approval of a drug through an NDA, applicants are required to submit to the FDA each patent that covers the applicant s product or FDA approved method of using this product. Those patents are then published in the FDA s Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an ANDA. Generally, an ANDA provides for marketing of a drug product that has the same active ingredients in the same strength(s), route of administration, and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. ANDA applicants are not required to conduct or submit results of pre-clinical or clinical tests to prove the safety or effectiveness of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as generic equivalents to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA s Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. A certification that the new product will not infringe the already approved product s listed patents or that such patents are invalid is called a Paragraph IV certification. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired. Alternatively, for a patent covering an approved method of use, an ANDA applicant may submit a statement to the FDA that the company is not seeking approval for the covered use.

If the ANDA applicant has submitted a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired. Federal law provides a period of five years following approval of a drug containing no previously approved active moiety, during which ANDAs for generic versions of those drugs cannot be submitted unless the submission contains a Paragraph IV challenge to a listed patent, in which case the submission may be made four years following the original product approval. Federal law provides for a period of three years of exclusivity

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following approval of a listed drug that contains previously approved active ingredients but is approved in a new dosage form, route of administration or combination, or for a new condition of use, the approval of which was required to be supported by new clinical trials conducted by or for the sponsor, during which the FDA cannot grant effective approval of an ANDA based on that listed drug. Both of the five-year and three-year exclusivity periods, as well as any unexpired patents listed in the Orange Book for the listed drug, can be extended by six months if the FDA grants the NDA sponsor a period of pediatric exclusivity based on studies submitted by the sponsor in response to a written request.

Section 505(b)(2) New Drug Applications

Most drug products (other than biological products) obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on the FDA s finding of safety and efficacy data for an existing product, or published literature, in support of its application.

Section 505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon certain preclinical or clinical studies conducted for an approved product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication for which the Section 505(b)(2) NDA applicant has submitted data.

To the extent that the Section 505(b)(2) applicant is relying on prior FDA findings of safety and efficacy, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus, approval of a Section 505(b)(2) NDA can be delayed until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) NDA applicant.

Orphan Drug Designation

Naglazyme, Aldurazyme, Kuvan and Firdapse have received orphan drug designations from the FDA. Orphan drug designation is granted by the FDA to drugs intended to treat a rare disease or condition, which for this program is defined as having a prevalence of less than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting a marketing application. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug.

Orphan drug designation does not shorten the regulatory review and approval process, nor does it provide any advantage in the regulatory review and approval process. However, if an orphan drug later receives approval for the indication for which it has designation, the relevant regulatory authority may not approve any other applications to market the same drug for the same indication, except in very limited circumstances, for seven years in the U.S. Although obtaining approval to market a product with orphan drug exclusivity may be advantageous, we cannot be certain:

- that we will be the first to obtain approval for any drug for which we obtain orphan drug designation;
- that orphan drug designation will result in any commercial advantage or reduce competition; or
- that the limited exceptions to this exclusivity will not be invoked by the relevant regulatory authority.

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Pediatric Information

Under the Pediatric Research Equity Act of 2007 (PREA), NDAs or BLAs or supplements to NDAs or BLAs must contain data to assess the safety and effectiveness of the drug for the claimed indication(s) in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan drug designation has been granted. The Best Pharmaceuticals for Children Act (BPCA), provides sponsors of NDAs with an additional six-month period of market exclusivity for all unexpired patent or non-patent exclusivity on all forms of the drug containing the active moiety, if the sponsor submits results of pediatric studies specifically requested by the FDA under BPCA within required timeframes. The Biologics Price Competition and Innovation Act, or BPCIA, provides sponsors of BLAs an additional six-month extension for all unexpired non-patent market exclusivity on all forms of the biological containing the active moiety pursuant to the BPCA if the conditions under the BPCA are met.

Accelerated Approval

Under the FDA s accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

Fast Track Designation

The FDA is required to facilitate the development and expedite the review of drugs that are intended for the treatment of a serious or life-threatening condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the fast track program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a fast track drug concurrent with or after the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor s request.

In addition to other benefits such as the ability to use surrogate endpoints and have greater interactions with the FDA, the FDA may initiate review of sections of a fast track drug s NDA or BLA before the application is complete. This rolling review is available if the applicant provides and the FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, the FDA s time period goal for reviewing an application does not begin until the last section of the NDA or BLA is submitted. Additionally, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Priority Review

Under the FDA policies, a drug candidate is eligible for priority review, or review within a six to eight month time frame from the time a complete NDA is submitted, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A fast track designated drug candidate would ordinarily meet the FDA s criteria for priority review. For biologics, priority

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review is further limited only for drugs intended to treat a serious or life-threatening disease relative to the currently approved products.

Post-Approval Regulatory Requirements

Following FDA approval, a product is subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of approved products, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the Internet.

Approved products may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, may require a submission to and approval by FDA before the change can be implemented. An NDA or BLA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA or BLA supplements as it does in reviewing NDAs and BLAs.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA or BLA. The FDA also may require post-marketing testing, known as Phase 4 testing, risk evaluation and mitigation strategies, and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control as well as the manufacture, packaging, and labeling procedures must continue to conform to cGMPs after approval. Drug and biological product manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA during which the agency inspects manufacturing facilities to access compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Patient Protection and Affordable Care Act of 2010

The Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (PPACA), is a sweeping measure intended to expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

The Biologics Price Competition and Innovation Act of 2009 (BPCIA), which was enacted as part of the PPACA, created an abbreviated approval pathway for biological products that are demonstrated to be biosimilar or interchangeable with an FDA-licensed reference biological product. Biosimilarity sufficient to reference a prior FDA-licensed product requires that there be no differences in conditions of use, route of administration, dosage form, and strength, and no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency. Biosimilarity must be shown through analytical studies, animal studies, and at least one clinical study, absent a waiver from the Secretary of Health and Human Services. In order to meet the higher hurdle of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. No biosimilar or interchangeable products have been approved under the BPCIA to date. Complexities associated with the larger, and often more complex, structures of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being evaluated by the FDA. A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product

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and no application for a biosimilar can be submitted for four years from the date of licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against a finding of interchangeability for other biologics for the same condition of use for the lesser of (i) one year after first commercial marketing of the first interchangeable biosimilar, (ii) eighteen months after the first interchangeable biosimilar is approved if there is not patent challenge, (iii) eighteen months after resolution of a lawsuit over the patents of the reference biologic in favor of the first interchangeable biosimilar applicant, or (iv) 42 months after the first interchangeable biosimilar s application has been approved if a patent lawsuit is ongoing within the 42-month period.

The PPACA also imposes a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The annual fee will be apportioned among the participating companies based on each company s sales of qualifying products to, or use by, certain U.S. government programs during the preceding year. Other provisions of the new law, which have varying effective dates, may also affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole . The law also revised the definition of average manufacturer price for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with health care practitioners.

In addition, drug manufacturers will be required to collect and report information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. The reported data will be posted in searchable form on a public web site. Failure to submit required information may result in civil monetary penalties. Although the statute requires reporting by March 31, 2013 of payments and other transfers of value made in calendar year 2012, the Centers for Medicare & Medicaid Services (CMS), has issued a final rule that will go into effect in April 2013 and will require manufacturers to begin collecting required information on August 1, 2013, with the first reports due March 31, 2014. Further, the PPACA amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them. In addition, the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the false claims laws.

Other Regulatory Requirements

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

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Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn are used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a company s products from reimbursement under government programs, criminal fines and imprisonment. Several states now require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to individual physicians in these states. Other states prohibit providing various other marketing-related activities. Still other states require the posting of information relating to clinical studies and their outcomes. In addition, California, Connecticut, Nevada, and Massachusetts require pharmaceutical companies to implement compliance programs or marketing codes. Currently, several additional states are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

Regulation in the European Union

Drugs are also subject to extensive regulation outside of the U.S. In the EU, for example, there is a centralized approval procedure that authorizes marketing of a product in all countries of the EU (which includes most major countries in Europe). If this procedure is not used, approval in one country of the EU can be used to obtain approval in another country of the EU under two simplified application processes, the mutual recognition procedure or the decentralized procedure, both of which rely on the principle of mutual recognition. After receiving regulatory approval through any of the European registration procedures, pricing and reimbursement approvals are also required in most countries.

A similar system for orphan drug designation exists in the EU. Naglazyme, Aldurazyme and Kuvan received orphan medicinal product designation by the European Committee for Orphan Medicinal Products. Orphan designation does not shorten the regulatory review and approval process for an orphan drug, nor does it give that drug any advantage in the regulatory review and approval process. However, if an orphan drug later receives approval for the indication for which it has designation, the relevant regulatory authority may not approve any other applications to market the same drug for the same indication, except in very limited circumstances, for ten years in the EU.

Anti-Corruption Legislation

The U.S. Foreign Corrupt Practices Act (FCPA), to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. Similar laws exist in other countries, such as the United Kingdom that restrict improper payments to public and private parties. Many countries have laws prohibiting these types of payments within the respective country. Historically, pharmaceutical companies have been the target of FCPA and other anti-corruption investigations and penalties.

Employees

As of January 4, 2013, we had 1,089 full-time employees, 457 of whom are in operations, 315 of whom are in research and development, 149 of whom are in sales and marketing and 168 of whom are in administration.

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We consider our employee relations to be good. Our employees are not covered by a collective bargaining agreement. We have not experienced employment related work stoppages.

Research and Development

For information regarding research and development expenses incurred during 2012, 2011 and 2010, see Item 7, *Management Discussion and Analysis of Financial Condition and Results of Operations Research and Development Expense* .

Geographic Area Financial Information

Our chief operating decision maker (*i.e.*, our chief executive officer) reviews financial information on a consolidated basis, for the purposes of allocating resources and evaluating financial performance. There are no segment managers who are held accountable by the chief operating decision maker, or anyone else, for operations, operating results and planning for levels or components below the consolidated unit level. Accordingly, we consider ourselves to have a single reporting segment and operating unit structure.

Net product revenues by geography are based on patients locations for Naglazyme, Kuvan and Firdapse, and are based on Genzyme s U.S. location for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties we earned on Genzyme s net sales are included in the U.S. as our transactions are with Genzyme.

The following table outlines net product revenues by geographic area (in thousands):

	Years Ended December 31,		
	2012	2011	2010
Net product revenues:			
United States	$ 249,745	$ 224,630	$ 196,979
Europe	108,138	100,348	90,321
Latin America	74,390	56,950	41,581
Rest of the World	64,224	55,719	40,820
Total net product revenues	$ 496,497	$ 437,647	$ 369,701

Total revenue generated outside the U.S. was $251.0 million, $217.1 million and $173.9 million, in the years ended December 31, 2012, 2011 and 2010, respectively.

The following table outlines non-monetary long-lived assets by geographic area (in thousands):

	Years Ended December 31,	
	2012	2011
Non-monetary long-lived assets:		
United States	$ 649,172	$ 652,207
International	80,067	80,459
Total long-lived assets	$ 729,252	$ 732,666

The decrease in non-monetary long-lived assets is primarily attributed to amortization of intangible assets and deprecation of property, plant and equipment, offset by capital expenditures.

Other Information

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. Our principal executive offices are located at 770 Lindaro Street, San Rafael, California 94901 and our telephone number is

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(415) 506-6700. Our annual reports on Form 10-K, quarterly reports on Form 10-Q, proxy statements, current reports on Form 8-K and amendments to those reports and statements filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, are available free of charge at *www.bmrn.com* as soon as reasonably practicable after we electronically file such reports with the U.S. Securities and Exchange Commission, or SEC. Such reports, statements and other information may be obtained by visiting the SEC s Public Reference Room at 100 F Street, NE, Washington, DC 20549 or by calling the SEC at 1-800-SEC-0330. Additionally, these reports are available at the SEC s website at *http://www.sec.gov*. Information contained in our website is not part of this or any other report that we file with or furnish to the SEC.

Item 1A. Risk Factors

An investment in our securities involves a high degree of risk. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the trading price of our securities to decline, and you may lose all or part of your investment.

If we fail to obtain or maintain regulatory approval to commercially market and sell our drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. Products distributed abroad are also subject to government regulation by international regulatory authorities. Naglazyme, Aldurazyme and Kuvan have received regulatory approval to be commercially marketed and sold in the U.S., EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU. Although we announced in November 2012 that our Phase 3 study of Vimizim , an enzyme replacement therapy for patients with MPS IVA (Morquio Syndrome), had met its primary endpoint, Vimizim has not received regulatory approval in the U.S., EU or any other jurisdiction and may never receive approval. Also, even if we receive priority review timelines from the FDA for Vimizim, there is no assurance that the FDA will comply with such timelines and there may be delays and ultimately the FDA may decide not to approve Vimizim.

As part of the recent reauthorization of PDUFA, new biologics are included in a new product review program intended to enhance FDA-sponsor communications to lead to greater first-cycle approval decisions. As part of this program, applications for new biologics are subject to either a 12-month standard or 8-month priority review period that begins from the date of application submission. However, since this is a new product review program and no products have completed this new review process, the priority review period may take longer than eight months and the standard review period may take longer than 12 months. Similarly, although the EMA has an accelerated approval process, the timelines mandated by the regulations are subject to the possibility of substantial delays.

In addition, the FDA and its international equivalents have substantial discretion over the approval process for pharmaceutical products. As such, these regulatory agencies may in the end not agree that we have demonstrated the requisite level of product safety and efficacy to grant approval and may require additional data. If we fail to obtain regulatory approval for our product candidates, including Vimizim, we will be unable to market and sell those drug products. Because of the risks and uncertainties in pharmaceutical development, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. We also rely on independent third-party contract research organizations, or CROs, to file

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some of our ex-U.S. and ex-EU marketing applications and important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, if the CRO elects to prioritize work on our projects below other projects or if there is any dispute or disruption in our relationship with our CROS, the filing of our applications may be delayed.

From time to time during the regulatory approval process for our products and our product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding the regulatory requirements for our development programs. To the extent appropriate, we accommodate the requests of the regulatory authorities and, to date, we have generally been able to reach reasonable accommodations and resolutions regarding the underlying issues. However, we are often unable to determine the outcome of such deliberations until they are final. If we are unable to effectively and efficiently resolve and comply with the inquiries and requests of the FDA and other non-U.S. regulatory authorities, the approval of our product candidates may be delayed and their value may be reduced.

After any of our products receive regulatory approval, they remain subject to ongoing regulation, which can impact, among other things product labeling, manufacturing practices, adverse event reporting, storage, expiration, distribution, advertising and promotion, and record keeping. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product s approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. Further, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA and comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, the value of our company and our operating results will be adversely affected. Additionally, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased.

If we fail to obtain or maintain orphan drug exclusivity for some of our products, our competitors may sell products to treat the same conditions and our revenues will be reduced.

As part of our business strategy, we intend to develop some drugs that may be eligible for FDA and EU orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the U.S. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available in the EU with a ten-year period of market exclusivity.

Because the extent and scope of patent protection for some of our drug products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have obtained orphan drug designation for certain of our products and product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties

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associated with developing pharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

We may face competition from biological products approved through an abbreviated regulatory pathway.

Our Naglazyme and Aldurazyme products, as well as certain of our product candidates, including Vimizim, are regulated by the FDA as biologics under the Federal Food, Drug and Cosmetics Act, or the FDC Act, and the Public Health Service Act. Biologics require the submission of a Biologics License Application (BLA), and approval by the FDA prior to being marketed in the U.S. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the PPACA, created a regulatory pathway for the abbreviated approval for biological products that are demonstrated to be biosimilar or interchangeable with an FDA-approved biological product. In order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Such biosimilars would reference biological products approved in the U.S. The law establishes a period of 12 years of data exclusivity for reference products, which protects the data in the original BLA by prohibiting sponsors of biosimilars from gaining FDA approval based in part on reference to data in the original BLA. Our products approved under BLAs, as well as products in development that may be approved under BLAs, could be reference products for such abbreviated BLAs.

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy preclinical and clinical trials are required and the results of the studies and trials are highly uncertain.

As part of the regulatory approval process, we must conduct, at our own expense, preclinical studies in the laboratory and clinical trials on humans for each product candidate. We expect the number of preclinical studies and clinical trials that the regulatory authorities will require will vary depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, the number and size of clinical trials required for approval increases based on the expected patient population that may be treated with a drug. We may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays in our ability to market any of our product candidates. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our drug products are safe and efficacious for use in the targeted human patients in order to receive regulatory approval for commercial sale.

Adverse or inconclusive clinical results would stop us from filing for regulatory approval of our product candidates. Additional factors that can cause delay or termination of our clinical trials include:

- slow or insufficient patient enrollment;
- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;

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adverse medical events or side effects in treated patients;

lack of effectiveness of the product candidate being tested; and

regulatory requests for additional clinical trials.

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party contract research organizations, or CROs, to perform most of our clinical studies and many important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs processes, methodologies or results were determined to be invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

If we continue to incur operating losses for a period longer than anticipated, we may be unable to continue our operations at planned levels and be forced to reduce our operations.

Since we began operations in March 1997, we have been engaged in very substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008 and 2010, we operated at a net loss in 2009, 2011 and 2012. Based upon our current plan for investments in research and development for existing and new programs, we expect to operate at a net loss for at least the next 12 months. Our future profitability depends on our marketing and selling of Naglazyme, Kuvan and Firdapse, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, either by ourselves or jointly with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.

Before we can begin commercial manufacture of our products, we, or our contract manufacturers, must obtain regulatory approval of our manufacturing facilities, processes and quality systems. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facilities in the U.S. have been approved by the FDA, the European Commission (EC), and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011has not yet been approved by the FDA or EMA. In addition, our third-party manufacturers facilities involved with the manufacture of Naglazyme, Kuvan, Firdapse and Aldurazyme have also been inspected and approved by various regulatory authorities.

Due to the complexity of the processes used to manufacture our products and product candidates, we may be unable to continue to pass or initially pass federal or international regulatory inspections in a cost effective manner. For the same reason, any potential third-party manufacturer of Naglazyme, Kuvan, Aldurazyme and Firdapse or our product candidates may be unable to comply with GMP regulations in a cost effective manner and may be unable to initially or continue to pass a federal or international regulatory inspection.

If we, or third-party manufacturers with whom we contract, are unable to comply with manufacturing regulations, we may be subject to fines, unanticipated compliance expenses, recall or seizure of our products, total or partial suspension of production and/or enforcement actions, including injunctions, and criminal or civil prosecution. These possible sanctions would adversely affect our financial results and financial condition.

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If we fail to obtain the capital necessary to fund our operations, our financial results and financial condition will be adversely affected and we will have to delay or terminate some or all of our product development programs.

As of December 31, 2012, we had cash, cash equivalents and short and long-term investments totaling $566.7 million. We may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise additional financing if we need such funds, we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

We expect to continue to spend substantial amounts of capital for our operations for the foreseeable future. The amount of capital we will need depends on many factors, including:

- our ability to successfully market and sell Naglazyme, Kuvan and Firdapse;
- Genzyme s ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress of research programs carried out by us;
- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc. that trigger related milestone payments;
- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and
- whether our convertible debt is converted to common stock in the future.

Moreover, our fixed expenses such as rent, license payments, interest expense and other contractual commitments are substantial and may increase in the future. These fixed expenses may increase because we may enter into:

additional licenses and collaborative agreements;

additional contracts for product manufacturing; and

additional financing facilities.

We may need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional securities may result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

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If we are unable to successfully develop and maintain manufacturing processes for our drug products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program.

Due to the complexity of manufacturing our products, we may not be able to manufacture drug products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at acceptable margins.

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities may require us to complete clinical trials to receive regulatory approval of any manufacturing improvements. Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of analytical and functional testing or may have to complete additional clinical studies. Also, if we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw materials and cell banks, malfunctions of internal information technology systems, and other events that cannot always be prevented or anticipated. Many of the processes include biological systems, which add significant complexity, as compared to chemical synthesis. We expect that, from time to time, consistent with biotechnology industry expectations, certain production lots will fail to produce product that meets our quality control release acceptance criteria. To date, our historical failure rates for all of our product programs, including Naglazyme, Aldurazyme and Vimizim, have been within our expectations, which are based on industry norms. If the failure rate increased substantially, we could experience increased costs, lost revenue, damage to customer relations, time and expense investigating the cause and, depending upon the cause, similar losses with respect to other lots or products. If problems are not discovered before the product is released to the market, recall and product liability costs may also be incurred.

In order to produce product within our time and cost parameters, we must continue to produce product within our expected success rate and yield expectations. Because of the complexity of our manufacturing processes, it may be difficult or impossible for us to determine the cause of any particular lot failure and we must effectively take corrective action in response to any failure in a timely manner.

Although we have entered into contractual relationships with third-party manufacturers to produce the active ingredient in Kuvan and Firdapse, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. We have contracts for the production of final product for Kuvan and Firdapse. We also rely on third-parties for portions of the manufacture of Naglazyme and Aldurazyme. If those manufacturers are unwilling or unable to fulfill their contractual obligations or satisfy demand outside of or in excess of the contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. Further, the availability of suitable contract manufacturing capacity at scheduled or optimum times is not certain.

In addition, our manufacturing processes subject us to a variety of federal, state and local laws and regulations governing the use, generation, manufacture, storage, handling and disposal of hazardous materials and wastes resulting from their use. We may incur significant costs in complying with these laws and regulations.

If we are unable to effectively address manufacturing issues, we may be unable to meet demand for our products and lose potential revenue, have reduced margins, or be forced to terminate a program.

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Our manufacturing facility for Naglazyme, Aldurazyme and Vimizim is located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair our ability to manufacture Naglazyme, Aldurazyme and Vimizim or our third-party manufacturer s ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme, Aldurazyme and Vimizim. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, and the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, floods, power loss and similar events. If any disaster were to occur, or any terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, our ability to manufacture Naglazyme, Aldurazyme and Vimizim, or to have Kuvan or Firdapse manufactured, could be seriously, or potentially completely impaired, and our commercialization efforts and revenue could be seriously impaired. The insurance that we carry, the inventory that we maintain and our risk mitigation plans may not be adequate to cover our losses resulting from disasters or other business interruptions.

Supply interruptions may disrupt our inventory levels and the availability of our products and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

Numerous factors could cause interruptions in the supply of our finished products, including:

- timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;
- labor interruptions;
- changes in our sources for manufacturing;
- the timing and delivery of shipments;
- our failure to locate and obtain replacement manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials.

Any interruption in the supply of finished products could hinder our ability to distribute finished products to meet commercial demand.

With respect to our product candidates, production of product is necessary to perform clinical trials and successful registration batches are necessary to file for approval to commercially market and sell product candidates. Delays in obtaining clinical material or registration batches could delay regulatory approval for our product candidates.

Because the target patient populations for our products are small, we must achieve significant market share and maintain high per-patient prices for our products to achieve profitability.

All of our products target diseases with small patient populations. As a result, our per-patient prices must be relatively high in order to recover our development and manufacturing costs and achieve profitability. For Naglazyme and Vimizim, if approved, we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in the disease populations are small, it is not only important to find patients who begin therapy to achieve significant market penetration of the product, but we also need to be able to maintain these patients on therapy for an extended period of time. Due to the expected costs of treatment for our products for genetic diseases, we may be unable to maintain or obtain sufficient market share at a price high enough to justify our product development efforts and

manufacturing expenses.

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If we fail to obtain an adequate level of coverage and reimbursement for our drug products by third-party payers, the sales of our drugs would be adversely affected or there may be no commercially viable markets for our products.

The course of treatment for patients using our products is expensive. We expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. We expect that most families of patients will not be capable of paying for this treatment themselves. There will be no commercially viable market for our products without coverage and reimbursement from third-party payers. Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our revenue and gross margins will be adversely affected.

Third-party payers, such as government or private health care insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis.

Reimbursement in the EU must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until reimbursement is approved. The timing to complete the negotiation process in each country is highly uncertain, and in some countries we expect that it may exceed 12 months.

For our future products, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable or our future revenues and gross margins may be adversely affected.

A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenue in these countries.

We make a significant portion of our international sales of Naglazyme through special access or named patient programs, which do not require full product approval. We expect to also utilize these programs for Vimizim. The specifics of the programs vary from country to country. Generally, special approval must be obtained for each patient. The approval normally requires an application or a lawsuit accompanied by evidence of medical need. Generally, the approvals for each patient must be renewed from time to time.

These programs are not well defined in some countries and are subject to changes in requirements and funding levels. Any change to these programs could adversely affect our ability to sell our products in those countries and delay sales. If the programs are not funded by the respective government, there could be insufficient funds to pay for all patients. Further, governments have in the past undertaken and may in the future undertake, unofficial measures to limit purchases of our products, including initially denying coverage for purchasers, delaying orders and denying or taking excessively long to approve customs clearance. Any such actions could materially delay or reduce our revenues from such countries.

Without the special access programs, we would need to seek full product approval to commercially market and sell our products. This can be an expensive and time-consuming process and may subject our products to additional price controls. Because the number of patients is so small in some countries, it may not be economically feasible to seek and maintain a full product approval, and therefore the sales in such country would be permanently reduced or eliminated. For all of these reasons, if the special access programs that we are currently using are eliminated or restricted, our revenues could be adversely affected.

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If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected.

Our competitors may develop, manufacture and market products that are more effective or less expensive than ours. They may also obtain regulatory approvals for their products faster than we can obtain them (including those products with orphan drug designation) or commercialize their products before we do. If we do not compete successfully, our revenue would be adversely affected, and we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Governmental and private third-party payers have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the U.S. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

International operations are also generally subject to extensive price and market regulations, and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or mandatory price cuts or reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed or threatened to impose revenue caps limiting the annual volume of sales of Naglazyme. To the extent that these caps are significantly below actual demand, our future revenues and gross margins may be adversely affected.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

Government health care reform could increase our costs, and would adversely affect our revenue and results of operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The PPACA is a sweeping measure intended to expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

Several provisions of the new law, which have varying effective dates, may affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole, and imposes a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The law also revised the definition of average manufacturer price for reporting purposes, which could increase the amount of the Medicaid drug

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rebates paid to states. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with health care practitioners. For example, beginning in 2013, drug manufacturers will be required to report information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties. Although the statute requires reporting by March 31, 2013 of payments and other transfers of value made in calendar year 2012, the CMS has issued a final rule that will go into effect in April 2013 and will require manufacturers to begin collecting required information on August 1, 2013 with the first reports due March 31, 2014.

The reforms imposed by the new law will significantly impact the pharmaceutical industry; however, the full effects of the PPACA cannot be known until these provisions are implemented and the CMS and other federal and state agencies issue applicable regulations or guidance. Moreover, in the coming years, additional changes could be made to governmental healthcare programs that could significantly impact the success of our products or product candidates. We will continue to evaluate the PPACA, as amended, the implementation of regulations or guidance related to various provisions of the PPACA by federal agencies, as well as trends and changes that may be encouraged by the legislation and that may potentially have an impact on our business over time. The cost of implementing more detailed record keeping systems and otherwise complying with these regulations could substantially increase our costs. The changes to the way our products are reimbursed by the CMS could reduce our revenues. Both of these situations could adversely affect our results of operations.

We face credit risks from customers that may adversely affect our results of operations.

Our product sales to government-owned or supported customers in various countries outside of the U.S. are subject to significant payment delays due to government funding and reimbursement practices. This has resulted and may continue to result in an increase in days sales outstanding due to the average length of time that we have accounts receivable outstanding. If significant changes were to occur in the reimbursement practices of these governments or if government funding becomes unavailable, we may not be able to collect on amounts due to us from these customers and our results of operations would be adversely affected.

If we are found in violation of federal or state fraud and abuse laws, we may be required to pay a penalty or be suspended from participation in federal or state health care programs, which may adversely affect our business, financial condition and results of operation.

We are subject to various federal and state health care fraud and abuse laws, including anti-kickback laws, false claims laws and laws related to ensuring compliance. The federal health care program anti-kickback statute makes it illegal for any person, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal health care programs, such as Medicare and Medicaid. Under federal government regulations, certain arrangements, or safe harbors, are deemed not to violate the federal anti-kickback statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability, although we seek to comply with these safe harbors. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal and state false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Under the Health Insurance Portability and Accountability Act of 1996, we

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also are prohibited from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer s products from reimbursement under government programs, criminal fines and imprisonment.

Many states have adopted laws similar to the federal anti-kickback statute, some of which apply to referral of patients for health care services reimbursed by any source, not just governmental payers. In addition, the state of California and several other states have passed laws that require pharmaceutical companies to comply with both the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the PhRMA Code on Interactions with Healthcare Professionals.

Neither the government nor the courts have provided definitive guidance on the application of some of these laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. While we believe we have structured our business arrangements to comply with these laws, it is possible that the government could allege violations of, or convict us of violating, these laws. If we are found in violation of one of these laws, we are required to pay a penalty or are suspended or excluded from participation in federal or state health care programs and our business, financial condition and results of operation may be adversely affected.

We conduct a significant amount of our sales and operations outside of the U.S., which subjects us to additional business risks that could adversely affect our revenue and results of operations.

A significant portion of the sales of Aldurazyme and Naglazyme and all of the sales of Firdapse are generated from countries other than the United States. Additionally, we have operations in several European countries, Brazil, other Latin American countries, Turkey and Asia. We expect that we will continue to expand our international operations in the future. International operations inherently subject us to a number of risks and uncertainties, including:

- changes in international regulatory and compliance requirements that could restrict our ability to manufacture, market and sell our products;
- political and economic instability;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;

financial risks such as longer payment cycles, difficulty collecting accounts receivable and exposure to fluctuations in foreign currency exchange rates; and

regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors and service providers activities that may fall within the purview of the Foreign Corrupt Practices Act.

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations.

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As we continue to expand our existing international operations, we may encounter new risks. For example, as we focus on building our international sales and distribution networks in new geographic regions, we must continue to develop relationships with qualified local distributors and trading companies. If we are not successful in developing and maintaining these relationships, we may not be able to grow sales in these geographic regions. These or other similar risks could adversely affect our revenue and profitability.

If we are unable to protect our proprietary technology, we may not be able to compete as effectively.

Where appropriate, we seek patent protection for certain aspects of our technology. Patent protection may not be available for some of the products we are developing. If we must spend significant time and money protecting or enforcing our patents, designing around patents held by others or licensing, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed.

The patent positions of biopharmaceutical products are complex and uncertain. The scope and extent of patent protection for some of our products and product candidates are particularly uncertain because key information on some of our product candidates has existed in the public domain for many years. The composition and genetic sequences of animal and/or human versions of Naglazyme, Aldurazyme, and many of our product candidates have been published and are believed to be in the public domain. The chemical structure of BH4 (the active ingredient in Kuvan) and 3,4-DAP (the active ingredient in Firdapse) have also been published. Publication of this information may prevent us from obtaining or enforcing patents relating to our products and product candidates, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

We own or have licensed patents and patent applications related to Naglazyme, Kuvan, Aldurazyme and Firdapse and certain of our product candidates, including Vimizim. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

With respect to pending patent applications, unless and until actually issued, the protective value of these applications is impossible to determine. We do not know whether our patent applications will result in issued patents.

Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or that they filed their application for a patent on a claimed invention before we did. Competitors may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors may also contest our patents by showing the patent examiner or a court that the invention was not original, was not novel or was obvious, for example. In litigation, a competitor could claim that our issued patents are not valid or are unenforceable for a number of reasons. If a court agrees, we would not be able to enforce that patent. We have no meaningful experience with competitors interfering with or challenging the validity or enforceability of our patents or patent applications.

Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs. We may not have the financial ability to sustain a patent infringement action, or it may not be financially reasonable to do so.

Receipt of a patent may not provide much, if any, practical protection. For example, if we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent

The recently enacted America Invents Act, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a first-to-invent system to a first-to-file system, and the implementation of new procedures that permit competitors to challenge our patents in the U.S. Patent Office after grant.

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In addition, competitors may also seek intellectual property protection for their technology. Due to the amount of intellectual property in our field of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. For example, if a patent holder believes our product infringes their patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe their intellectual property, we would face a number of issues, including the following:

- Defending a lawsuit, which takes significant time and resources and can be very expensive.
- If a court decides that our product infringes a competitor s intellectual property, we may have to pay substantial damages.
- With respect to patents, in addition to requiring us to pay substantial damages, a court may prohibit us from making, selling, offering to sell, importing or using our product unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, it may not be available on commercially reasonable terms. For example, we may have to pay substantial royalties or grant cross licenses to our patents and patent applications.
- We may need to redesign our product so it does not infringe the intellectual property rights of others.
- Redesigning our product so it does not infringe the intellectual property rights of competitors may not be possible or could require substantial funds and time.

It is also unclear whether our trade secrets are adequately protected. Our employees, consultants or contractors may unintentionally or willfully disclose trade secrets to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, as with patent litigation, is expensive and time consuming, requires significant resources and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets. Furthermore, our competitors may independently develop equivalent knowledge, methods and know-how, in which case we would not be able to enforce our trade secret rights against such competitors.

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations.

If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or may be prohibited from making, using, importing, offering to sell or selling products requiring these licenses or rights. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. If we are not able to resolve such disputes and obtain the licenses or rights we need, we may not be able to develop or market our products.

If our Manufacturing, Marketing and Sales Agreement (MMS Agreement) with Genzyme were terminated, we could be prevented from continuing to commercialize Aldurazyme or our ability to successfully commercialize Aldurazyme would be delayed or diminished.

Either party may terminate the Manufacturing, Marketing and Sales Agreement (MMS Agreement), between Genzyme and us related to Aldurazyme for specified reasons, including if the other party is in material breach of the MMS, has experienced a change of control, as such term is defined in the MMS agreement, or has declared bankruptcy and also is in breach of the MMS. Although we are not currently in breach of the MMS, there is a risk that either party could breach the MMS in the future. Either party may also terminate the MMS upon one year prior written notice for any reason.

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in BioMarin/Genzyme LLC, or the LLC, to the non-breaching party, and the non-breaching party will pay a specified buyout

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amount for the breaching party s interest in Aldurazyme and in the LLC. If we are the breaching party, we would lose our rights to Aldurazyme and the related intellectual property and regulatory approvals. If the MMS Agreement is terminated without cause, the non-terminating party would have the option, exercisable for one year, to buy out the terminating party s interest in Aldurazyme and in the LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the LLC will be dissolved. In the event of termination of the buyout option without exercise by the non-terminating party as described above, all right and title to Aldurazyme is to be sold to the highest bidder, with the proceeds to be split between Genzyme and us in accordance with our percentage interest in the LLC.

If the MMS Agreement is terminated by either party because the other party declared bankruptcy, the terminating party would be obligated to buy out the other party and would obtain all rights to Aldurazyme exclusively. If the MMS Agreement is terminated by a party because the other party experienced a change of control, the terminating party shall notify the other party, the offeree, of its intent to buy out the offeree s interest in Aldurazyme and the LLC for a stated amount set by the terminating party at its discretion. The offeree must then either accept this offer or agree to buy the terminating party s interest in Aldurazyme and the LLC on those same terms. The party who buys out the other party would then have exclusive worldwide rights to Aldurazyme. The Amended and Restated Collaboration Agreement between us and Genzyme will automatically terminate upon the effective date of the termination of the MMS Agreement and may not be terminated independently from the MMS Agreement.

If we were obligated, or given the option, to buy out Genzyme s interest in Aldurazyme and the LLC, and thereby gain exclusive rights to Aldurazyme, we may not have sufficient funds to do so and we may not be able to obtain the financing to do so. If we fail to buy out Genzyme s interest, we may be held in breach of the agreement and may lose any claim to the rights to Aldurazyme and the related intellectual property and regulatory approvals. We would then effectively be prohibited from developing and commercializing Aldurazyme. If this happened, not only would our product revenues decrease, but our share price would also decline.

Based on our strategic alliance with Merck Serono, unless Merck Serono opts in to the PEG-PAL program, we will not realize any cost sharing for the development expenses, development milestones, or royalties for ex-U.S. sales.

In May 2005, we entered into an agreement with Merck Serono for the further development and commercialization of Kuvan (and any other product containing 6R-BH4) and PEG-PAL for PKU. Pursuant to that agreement, we received development milestones on Kuvan and receive royalties on sales by Merck Serono. Additionally, we may be entitled to development milestones and royalties related to PEG-PAL. However, Merck Serono has opted out of the PEG-PAL development program. Unless and until it elects to opt in, it is not obligated to pay any of the milestones related to the program or to reimburse us for any of the development costs. Additionally, even though Merck Serono has opted out, we do not have any right to commercialize PEG-PAL outside of the U.S. and Japan or to grant anyone else such rights.

Merck Serono may elect to opt in at any time. If Merck Serono opts in to the PEG-PAL development program before the unblinding of the first Phase 3 trial for PEG-PAL, it must pay 75% of the Phase 3 costs incurred prior to the opt-in and the $7,000,000 Phase 3 initiation milestone, if the trial has started. If it opts in after unblinding of the first Phase 3 trial for PEG-PAL, it must pay 100% of the Phase 3 costs incurred prior to the opt-in and the $7,000,000 Phase 3 initiation milestone. Additionally, in all cases after it opts in to the PEG-PAL development program, Merck Serono would be obligated to pay one half of future development costs under the agreement and any further milestones due under the agreement. If Merck Serono does not opt in, it will not have the right to use any of the clinical or other independently developed data.

We cannot determine when or if Merck Serono will opt in to the PEG-PAL development program. If Merck Serono does not opt in, we will not receive any milestones under the agreement nor will there be any sales outside of the U.S. or Japan generating revenue from royalties or otherwise.

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If we fail to compete successfully with respect to acquisitions, joint ventures or other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

Our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our product programs have been acquired through acquisitions, such as BMN-701 and BMN-673 and several of our product programs have been developed through licensing or collaborative arrangements, such as Naglazyme, Aldurazyme, Kuvan and Firdapse. These collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. Our future success will depend, in part, on our ability to identify additional opportunities and to successfully enter into partnering or acquisition agreements for those opportunities. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Several pharmaceutical and biotechnology companies have already established themselves in the field of genetic diseases. These companies have already begun many drug development programs, some of which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

If generic manufacturers use litigation and regulatory means to obtain approval for generic versions of Kuvan, our revenue and results of operations would be adversely affected.

The Hatch Waxman Act permits the FDA to approve abbreviated new drug applications, or ANDAs, for generic versions of branded drugs. We refer to this process as the ANDA process . The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient for the same uses but does not generally require the conduct and submission of clinical efficacy studies for that product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product based on pharmacokinetic studies. Pursuant to the Hatch Waxman Act, companies were able to file an ANDA application for the active ingredient in Kuvan at any time after December 2011. At present, we have no information that any other party has filed or has conducted the bioequivalency study necessary to file an ANDA for Kuvan.

The Hatch Waxman Act requires an applicant for a drug that relies, at least in part, on our data regarding the safety and efficacy of Kuvan, to notify us of their application and potential infringement of our patents listed in the FDA s Approved Drug Products with Therapeutic Equivalence Evaluations (Orange Book). Upon receipt of a notice alleging that our patents listed in the Orange Book are invalid or not infringed by the proposed competitor product (paragraph iv notice), we would have 45 days to bring a patent infringement suit in federal district court against the company seeking approval for its product. The discovery, trial and appeals process in such suits can take several years. If we commence such a suit alleging infringement of one or more of our Orange Book listed patents within 45 days from receipt of the paragraph iv notice, the Hatch Waxman Act provides a 30-month stay on the FDA s approval of the competitor s application. If the litigation is resolved in favor of the applicant or the challenged patent expires during the 30-month stay period, the stay is lifted and the FDA s review of the application may be completed. Such litigation is often time-consuming, costly and may result in competition if

such patent(s) are not upheld or if the competitor does not infringe such patent(s). However, generic versions of Kuvan would be prohibited until the expiration of orphan drug exclusivity in December 2014 or June 2015 if we receive pediatric exclusivity.

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The filing of an ANDA application in respect to Kuvan could have an adverse impact on our stock price and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan were not upheld in litigation or if the generic competitor is found to not infringe these patents, the resulting generic competition following the expiration of orphan exclusivity would have a material adverse effect on our revenue and results of operations.

If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we publicly announce the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We depend upon our key personnel and our ability to attract and retain employees.

Our future growth and success will depend in large part on our continued ability to attract, retain, manage and motivate our employees. The loss of the services of any member of our senior management or the inability to hire or retain experienced management personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of one or more of our senior executive officers could be detrimental to us if we do not have an adequate succession plan or if we cannot recruit suitable replacements in a timely manner. While our senior executive officers are parties to employment agreements with us, these agreements do not guarantee that they will remain employed with us in the future. In addition, in many cases, these agreements do not restrict our senior executive officers ability to compete with us after their employment is terminated. The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Due to this intense competition, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. If we are unsuccessful in our recruitment and retention efforts, our business may be harmed.

Our success depends on our ability to manage our growth.

Product candidates that we are currently developing or may acquire in the future may be intended for patient populations that are significantly larger than any of MPS I, MPS VI, PKU or LEMS. In order to continue development and marketing of these products, if approved, we will need to significantly expand our operations. To manage expansion effectively, we need to continue to develop and improve our research and development capabilities, manufacturing and quality capacities, sales and marketing capabilities, financial and administrative systems and standard processes for global operations. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and may increase our exposure to regulatory and corruption risks and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third-parties.

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Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.

Even if our drug products are approved, if doctors elect a course of treatment which does not include our drug products, this decision would reduce demand for our drug products and adversely affect revenues. For example, if gene therapy becomes widely used as a treatment of genetic diseases, the use of enzyme replacement therapy, such as Naglazyme and Aldurazyme in MPS diseases, could be greatly reduced. Changes in treatment method can be caused by the introduction of other companies products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

If product liability lawsuits are successfully brought against us, we may incur substantial liabilities.

We are exposed to the potential product liability risks inherent in the testing, manufacturing and marketing of human pharmaceuticals. We maintain insurance against product liability lawsuits for commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of Naglazyme, Kuvan, Aldurazyme and Firdapse, or our clinical trials for PEG-PAL, Vimizim, BMN-701, BMN-673 or BMN-111 for which our insurance coverage may not be adequate.

The product liability insurance we will need to obtain in connection with the commercial sales of our product candidates if and when they receive regulatory approval may be unavailable in meaningful amounts or at a reasonable cost. In addition, while we continue to take what we believe are appropriate precautions, we may be unable to avoid significant liability if any product liability lawsuit is brought against us. If we are the subject of a successful product liability claim that exceeds the limits of any insurance coverage we obtain, we may incur substantial charges that would adversely affect our earnings and require the commitment of capital resources that might otherwise be available for the development and commercialization of our product programs.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy, or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents could harm our ability to operate our business effectively. Our ability to manage and maintain our inventory and internal reports, to manufacture and ship our products to customers and timely invoice them depends significantly on our enterprise resource planning, production management, and other information systems. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data could require significant capital investments to remediate any such failure, problem or breach, all of which could adversely affect our business, financial condition and results of operations.

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Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases on to our customers due to the process by which health care providers are reimbursed for our products by the government. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the value of our investments and our ability to liquidate our investments in order to fund our operations. We purchase or enter into a variety of financial instruments and transactions, including investments in commercial paper, the extension of credit to corporations, institutions and governments and hedging contracts. If any of the issuers or counter parties to these instruments were to default on their obligations, it could materially reduce the value of the transaction and adversely affect our cash flows.

For the year ended December 31, 2012, approximately 4% of our net product revenues were from the Southern European countries of Italy, Spain, Portugal and Greece. Approximately 8% of our total accounts receivable as of December 31, 2012 related to such countries and we have included an allowance for doubtful accounts for certain accounts receivable from Greece. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected.

Interest rates and the ability to access credit markets could also adversely affect the ability of our customers/distributors to purchase, pay for and effectively distribute our products. Similarly, these macroeconomic factors could affect the ability of our contract manufacturers, sole-source or single-source suppliers to remain in business or otherwise manufacture or supply product. Failure by any of them to remain a going concern could affect our ability to manufacture products.

Risks Related to Ownership of Our Securities

Our stock price may be volatile, and an investment in our stock could suffer a decline in value.

Our valuation and stock price since the beginning of trading after our initial public offering have had no meaningful relationship to current or historical earnings, asset values, book value or many other criteria based on conventional measures of stock value. The market price of our common stock will fluctuate due to factors including:

- product sales and profitability of Naglazyme, Aldurazyme, Kuvan and Firdapse;
- manufacture, supply or distribution of Naglazyme, Aldurazyme, Kuvan and Firdapse;
- progress of our product candidates through the regulatory process;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;
- government regulatory action affecting our product candidates or our competitors drug products in both the U.S. and non U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;

economic conditions in the U.S. or abroad;

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broad market fluctuations in the U.S., the EU or in other parts of the world;

actual or anticipated fluctuations in our operating results; and

changes in our assessments or financial estimates by securities analysts.

In the past, following periods of large price declines in the public market price of a company s securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management s attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. In addition, the current decline in the financial markets and related factors beyond our control, including the credit and mortgage crisis in the U.S. and worldwide, may cause our stock price to decline rapidly and unexpectedly.

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our certificate of incorporation providing that stockholders meetings may only be called by our Board of Directors and provisions in our bylaws providing that the stockholders may not take action by written consent and requiring that stockholders that desire to nominate any person for election to our Board of Directors or to make any proposal with respect to business to be conducted at a meeting of our stockholders be submitted in appropriate form to our Secretary within a specified period of time in advance of any such meeting. Additionally, our Board of Directors has the authority to issue additional shares of preferred stock and to determine the terms of those shares of stock without any further action by our stockholders, which would allow our Board of Directors to implement a stockholder rights plan without approval by our stockholders. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, our Board of Directors approves the transaction. Our Board of Directors may use these provisions to prevent changes in the management and control of our company. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

The following table contains information about our current significant owned and leased properties:

Location	Approximate Square Feet	Use	Lease Expiration Date
San Rafael facility, San Rafael, California	120,400	Corporate headquarters, office	2022
Several locations in Novato, California	273,000	Office, laboratory and warehouse	2011-2020
Galli Drive facility, Novato, California	91,500	Clinical and commercial manufacturing and laboratory	NA: owned property
Bel Marin Keys facility, Novato, California	83,900	Technical operations, finance, administration, and laboratory	NA: owned property
Shanbally facility, Cork, Ireland	142,000	Manufacturing	NA: owned property

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Our administrative office space and plans to develop additional space are expected to be adequate for the foreseeable future. In addition to the above, we also maintain small offices in a variety of locations around the world. We believe that, to the extent required, we will be able to lease or buy additional facilities at commercially reasonable rates. We plan to use contract manufacturing when appropriate to provide product for both clinical and commercial requirements until such time as we believe it prudent to develop additional in-house clinical and/or commercial manufacturing capacity.

Item 3. Legal Proceedings

We have no material legal proceedings pending.

Item 4. Mine Safety Disclosures

Not applicable

Part II

Item 5. Market for Registrant s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Our common stock is listed under the symbol BMRN on the NASDAQ Global Select Market. The following table sets forth the range of high and low quarterly closing sales prices for our common stock for the periods noted, as reported by NASDAQ.

		Prices	
Year	**Period**	**High**	**Low**
2012	First Quarter	$ 38.34	$ 33.68
2012	Second Quarter	$ 39.58	$ 32.13
2012	Third Quarter	$ 43.30	$ 37.02
2012	Fourth Quarter	$ 50.17	$ 36.78
2011	First Quarter	$ 28.29	$ 23.46
2011	Second Quarter	$ 28.46	$ 24.93
2011	Third Quarter	$ 31.87	$ 24.02
2011	Fourth Quarter	$ 35.38	$ 30.07

On February 15, 2013, the last reported sale price on the NASDAQ Global Select Market for our common stock was $56.28. We have never paid any cash dividends on our common stock and we do not anticipate paying cash dividends in the foreseeable future.

Issuer Purchases of Equity Securities

We did not make any purchases of our common stock during the year ended December 31, 2012.

Holders

As of February 15, 2013, there were 54 holders of record of 126,101,610 outstanding shares of our common stock. Additionally, on such date, options to acquire 13.7 million shares of our common stock were outstanding.

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Performance Graph

The following is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language in such filing.

The following graph shows the value of an investment of $100 on December 31, 2007 in BioMarin common stock, the NASDAQ Composite Index (U.S.) and the NASDAQ Biotechnology Index. All values assume reinvestment of the pretax value of dividends paid by companies included in these indices and are calculated as of December 31 of each year. Our common stock is traded on the NASDAQ Global Select Market and is a component of both the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.

* $100 invested on December 31, 2007 in stock or index, including reinvestment of dividends.

	Fiscal Year Ending December 31,					
	2007	**2008**	**2009**	**2010**	**2011**	**2012**
BioMarin Pharmaceutical Inc.	100.00	50.28	53.14	76.07	97.12	138.98
NASDAQ Composite Index	100.00	59.03	82.25	97.32	98.63	110.78
NASDAQ Biotechnology Index	100.00	93.40	103.19	113.89	129.12	163.33

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Item 6. Selected Consolidated Financial Data

The information set forth below for the five years ended December 31, 2012 is not necessarily indicative of results of future operations, and should be read in conjunction with Item 7, *Management s Discussion and Analysis of Financial Condition and Results of Operations* and the Consolidated Financial Statements and related notes thereto included in Item 8 of this Annual Report on Form 10-K to fully understand factors that may affect the comparability of the information presented below:

	Years Ended December 31, (In thousands of U.S. dollars, except for per share data)				
	2012	2011	2010	2009	2008
Consolidated statements of operations data:					
REVENUES:					
Net product revenues	$ 496,497	$ 437,647	$ 369,701	$ 315,721	$ 251,851
Collaborative agreement revenues	1,955	468	682	2,379	38,907
Royalty and license revenues	2,271	3,243	5,884	6,556	5,735
Total revenues	500,723	441,358	376,267	324,656	296,493
OPERATING EXPENSES:					
Cost of sales (excludes amortization of certain acquired intangible assets)	91,830	84,023	70,285	65,909	52,509
Research and development	302,218	214,374	147,309	115,116	93,291
Selling, general and administrative	198,173	175,423	151,723	124,290	106,566
Intangible asset amortization and contingent consideration	18,717	1,428	6,406	2,914	4,371
Total operating expenses	610,938	475,248	375,723	308,229	256,737
INCOME (LOSS) FROM OPERATIONS	(110,215)	(33,890)	544	16,427	39,756
Equity in the loss of BioMarin/Genzyme LLC	(1,221)	(2,426)	(2,991)	(2,594)	(2,270)
Interest income	2,584	2,934	4,112	5,086	16,695
Interest expense	(7,639)	(8,409)	(10,818)	(14,404)	(16,394)
Debt conversion expense	0	(1,896)	(13,728)	0	0
Impairment loss on equity investments	0	0	0	(5,848)	(4,056)
Net gain from sale of investments	0	0	902	1,585	0
Other income (expense)	(1,787)	60	489	314	(307)
INCOME (LOSS) BEFORE INCOME TAXES	(118,278)	(43,627)	(21,490)	566	33,424
Provision for (benefit from) income taxes	(3,931)	10,209	(227,309)	1,054	2,593
NET INCOME (LOSS)	$ (114,347)	$ (53,836)	$ 205,819	$ (488)	$ 30,831
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.95)	$ (0.48)	$ 2.00	$ (0.00)	$ 0.31
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.95)	$ (0.48)	$ 1.73	$ (0.00)	$ 0.29
Weighted average common shares outstanding, basic	120,271	112,122	103,093	100,271	98,975
Weighted average common shares outstanding, diluted	120,271	112,122	125,674	100,271	103,572

	December 31, (in thousands)				
	2012	2011	2010	2009	2008
Consolidated balance sheet data:					
Cash, cash equivalents and investments	$ 566,731	$ 289,477	$ 402,283	$ 470,526	$ 561,425
Total current assets	743,462	469,802	504,260	467,727	737,696
Total assets	1,601,643	1,305,709	1,262,623	917,163	906,695
Long-term liabilities, net of current portion	415,447	438,536	461,522	516,824	499,939
Total stockholders equity	1,015,763	773,048	717,257	322,185	276,675

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You should read the following tables presenting our unaudited quarterly results of operations in conjunction with the Consolidated Financial Statements and related notes contained elsewhere in this Annual Report on Form 10-K. We have prepared this unaudited information on the same basis as our audited Consolidated Financial Statements. Our quarterly operating results have fluctuated in the past and may continue to do so in the future as a result of a number of factors, including, but not limited to, the timing and nature of research and development activities.

	Three Months Ended (In thousands, except per share data, unaudited)			
	March 31,	June 30,	September 30,	December 31,
2012:				
Total revenue	$ 116,649	$ 124,019	$ 128,117	$ 131,938
Net loss	(23,972)	(32,006)	(5,357)	(53,012)
Net loss per share, basic and diluted	(0.21)	(0.27)	(0.04)	(0.43)
2011:				
Total revenue	$ 109,456	$ 110,631	$ 113,425	$ 107,846
Net loss	(4,371)	(5,077)	(17,653)	(26,735)
Net loss per share, basic and diluted	(0.04)	(0.05)	(0.16)	(0.23)

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Item 7. Management s Discussion and Analysis of Financial Condition and Results of Operations

The following discussion of our financial condition and results of operations should be read in conjunction with our Consolidated Financial Statements and notes to those statements included elsewhere in this Annual Report on Form 10-K.

Overview

We develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products.

Key components of our results of operations include the following (in millions):

	Years Ended December 31,		
	2012	2011	2010
Total net product revenues	$ 496.5	$ 437.6	$ 369.7
Cost of sales	91.8	84.0	70.3
Research and development expense	302.2	214.4	147.3
Selling, general and administrative expense	198.2	175.4	151.7
Intangible asset amortization and contingent consideration	18.7	1.4	6.4
Provision for (benefit from) income taxes	(3.9)	10.2	(227.3)
Net income (loss)	(114.3)	(53.8)	205.8
Stock-based compensation expense	48.0	43.8	37.5

See *Results of Operations* below for a discussion of the detailed components and analysis of the amounts above.

Our product portfolio is comprised of four approved products and multiple investigational product candidates. Our approved products are Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Firdapse (amifampridine phosphate) and Aldurazyme (laronidase).

Naglazyme, a recombinant form of N-acetylgalactosamine 4-sulfatase indicated for patients with mucopolysaccharidosis VI (MPS VI), a debilitating life-threatening genetic disease for which no other drug treatment currently exists and is caused by the deficiency of arylsufatase B, received marketing approval in the U.S. in May 2005, in the EU in January 2006 and subsequently in other countries. Naglazyme net product revenues for the year ended December 31, 2012, totaled $257.0 million, compared to $224.9 million and $192.7 million, respectively, for the years ended December 31, 2011 and 2010.

Kuvan was granted marketing approval for the treatment of phenylketonuria (PKU) in the U.S. and in the EU in December 2007 and December 2008, respectively. Our Kuvan net product revenues for the year ended December 31, 2012 totaled $143.1 million, compared to $116.8 million and $99.4 million, respectively, for the years ended December 31, 2011 and 2010.

In December 2009, the European Medicines Agency granted marketing approval for Firdapse, a proprietary form of 3-4-diaminopyridine (amifampridine phosphate), for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS). We launched this product on a country by country basis in the EU beginning in April 2010. Firdapse net product revenues for the year ended December 31, 2012 totaled $14.2 million, compared to $13.1 million and $6.4 million, respectively, for the years ended December 31, 2011 and 2010.

Aldurazyme (laronidase), which was developed in collaboration with Genzyme Corporation (Genzyme), was approved in 2003 for marketing in the U.S., the EU and subsequently in other countries for patients with

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

mucopolysaccharidosis I (MPS I). Our Aldurazyme net product revenues for the year ended December 31, 2012 totaled $82.2 million, compared to $82.8 million and $71.2 million, respectively for the years ended December 31, 2011 and 2010.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including:

- Vimizim™, formerly referred to as GALNS, an enzyme replacement therapy for the treatment of mucopolysaccharidosis Type IV or Morquio Syndrome Type A, a lysosomal storage disorder;
- PEG-PAL, an enzyme substitution therapy for the treatment of PKU;
- BMN-701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder;
- BMN-673, an orally available poly-ADP ribose polymerase inhibitor for the treatment of patients with certain cancers; and
- BMN-111, a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism.

We are conducting preclinical development of several other product candidates for genetic and other metabolic diseases, including BMN-190 for late infantile neuronal ceroid lipofuscinosis (LINCL), a lysosomal storage disorder primarily affecting the brain.

Cost of sales includes raw materials, personnel and facility and other costs associated with manufacturing Naglazyme and Aldurazyme at our production facility in Novato, California. Cost of sales also includes third-party manufacturing costs for the production of the active ingredient in Kuvan and Firdapse and third-party production costs related to final formulation and packaging services for all products and cost of royalties payable to third-parties for all products.

Research and development includes costs associated with the research and development of product candidates and post-marketing research commitments related to our approved products. These costs primarily include preclinical and clinical studies, personnel and raw materials costs associated with manufacturing product candidates, quality control and assurance and regulatory costs.

Selling, general and administrative expense primarily includes expenses associated with the commercialization of approved products and general and administrative costs to support our operations. These expenses include: product marketing and sales operations personnel; corporate facility operating expenses; information technology expenses and depreciation; and core corporate support functions, including human resources, finance and legal, and other external corporate costs such as insurance, legal fees and other professional services.

Intangible asset amortization and contingent consideration includes amortization expense related to our finite-lived intangible assets associated with marketing rights in the EU for Firdapse, impairment losses (if any) on intangible assets and changes in the fair value of contingent acquisition consideration payable. Changes in fair value can result from changes in estimated probability adjustments, changes in estimated timing of when a milestone may be achieved, changes in assumed discount periods and rates and passage of time.

Our cash, cash equivalents, short-term investments and long-term investments totaled $566.7 million as of December 31, 2012, compared to $289.5 million as of December 31, 2011. We have historically financed our operations primarily through the issuance of common stock and convertible debt and by relying on equipment and other commercial financing. We will be highly dependent on our net product revenue to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing. Further, depending on market conditions, our

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financial position and performance and other factors, we may in the future choose to use a portion of our cash or cash equivalents to repurchase our convertible debt or other securities. See *Financial Position, Liquidity and Capital Resources* below for a further discussion of our liquidity and capital resources.

Critical Accounting Policies and Estimates

In preparing our Consolidated Financial Statements in accordance with accounting principles generally accepted in the U.S. and pursuant to the rules and regulations promulgated by the SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/(loss) and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions. On a regular basis, we evaluate our assumptions, judgments and estimates. We also discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors.

We believe that the assumptions, judgments and estimates involved in the accounting for business combinations, contingent acquisition consideration payable, income taxes, long-lived assets, revenue recognition and inventory have the greatest impact on our Consolidated Financial Statements, so we consider these to be our critical accounting policies. Historically, our assumptions, judgments and estimates relative to our critical accounting policies have not differed materially from actual results.

Business Combinations

We allocate the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development (IPR&D). In connection with the purchase price allocations for acquisitions, we estimate the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate.

Although we believe the assumptions and estimates made are reasonable, they are based in part on historical experience and information obtained from the management of the acquired businesses and are inherently uncertain. Examples of critical estimates in valuing any contingent acquisition consideration issued or which may be issued and the intangible assets we have acquired or may acquire in the future include but are not limited to:

- the feasibility and timing of achievement of development, regulatory and commercial milestones;
- expected costs to develop the in-process research and development into commercially viable products; and
- future expected cash flows from product sales.

Unanticipated events and circumstances may occur which may affect the accuracy or validity of such assumptions, estimates or actual results.

Valuation of Contingent Acquisition Consideration Payable

Each period we reassess the fair value of the contingent acquisition consideration payable associated with certain acquisitions and record increases in the fair value as contingent consideration expense and record decreases in the fair value as a reduction of contingent consideration expense. Increases or decreases in the fair value of the contingent acquisition consideration payable can result from changes in estimated probability adjustments with respect to regulatory approval, changes in the assumed timing of when milestones are likely to

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be achieved and changes in assumed discount periods and rates. Significant judgment is employed in determining the appropriateness of these assumptions each period. Accordingly, future business and economic conditions, as well as changes in any of the assumptions described in the accounting for business combinations above can materially impact the amount of contingent consideration expense that we record in any given period.

Income Taxes

Our Consolidated Balance Sheets reflect net deferred tax assets that primarily represent the tax benefit of net operating loss carryforwards and credits and timing differences between book and tax recognition of certain revenue and expense items, net of a valuation allowance. When it is more likely than not that all or some portion of deferred tax assets may not be realized, we establish a valuation allowance for the amount that may not be realized. Each quarter, we evaluate the need to retain all or a portion of the valuation allowance on our net deferred tax assets. Our evaluation considers historical earnings, estimated future taxable income and ongoing prudent and feasible tax planning strategies. Adjustments to the valuation allowance increase or decrease net income (loss) in the period such adjustments are made. If our estimates require adjustments, it could have a significant impact on our Consolidated Financial Statements.

We continually review the adequacy and necessity of the valuation allowance. If it is more likely than not that we would not realize the deferred tax benefits, then all or a portion of the valuation allowance may need to be re-established. Changes in tax laws and rates could also affect recorded deferred tax assets in the future. Management is not aware of any such changes that would have a material effect on our Consolidated Financial Statements.

Impairment of Long-Lived Assets

Our long-lived assets include our investment in BioMarin/Genzyme LLC, long-term investments, property, plant and equipment, intangible assets and goodwill. We regularly review long-lived assets for impairment. The recoverability of our debt and equity investments is measured by available external market data, including quoted prices on public exchanges and other relevant information. If the carrying amount of the asset is not recoverable, an impairment loss is recorded for the amount that the carrying value of the asset exceeds its fair value.

The recoverability of long-lived assets, other than goodwill, indefinite-lived intangible assets and our long-term investments is measured by comparing the asset s carrying amount to the expected undiscounted future cash flows that the asset is expected to generate. Determining whether an impairment has occurred typically requires various estimates and assumptions, including determining which cash flows are directly related to the potentially impaired asset, the useful life over which cash flows will occur, their amount, and the asset s residual value, if any. In turn, measurement of an impairment loss requires a determination of fair value, which is based on the best information available. We use internal cash flow estimates, quoted market prices when available and independent appraisals as appropriate to determine fair value. We derive the required cash flow estimates from our historical experience and our internal business plans and apply an appropriate discount rate.

The recoverability of the carrying value of buildings, leasehold improvements for our facilities and equipment will depend on the successful execution of our business initiatives and our ability to earn sufficient returns on our approved products and product candidates. We continually monitor events and changes in circumstances that could indicate carrying amounts of our fixed assets may not be recoverable. When such events or changes in circumstances occur, we assess recoverability by determining whether the carrying value of such assets will be recovered through the undiscounted expected future cash flows. If the future undiscounted cash flows are less than the carrying amount of these assets, we recognize an impairment loss based on the excess of the carrying amount over the fair value of the assets. Based on management s current estimates, we expect to recover the carrying value of such assets.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

We have recorded intangible assets, primarily related to IPR&D, and goodwill as part of our recognition and measurement of assets acquired and liabilities assumed in conjunction with our business combinations. Goodwill and intangible assets determined to be indefinite-lived assets are not amortized, but are required to be reviewed annually for impairment or more frequently if events and circumstances indicate that the carrying value may not be recoverable. We perform our annual impairment test of indefinite-lived intangible assets in the fourth quarter of each fiscal year and in between annual tests if we become aware of any events or changes in circumstances that would indicate a reduction in the fair value of the assets below their carrying values. As of December 31, 2012, we had $63.7 million of indefinite-lived assets related to IPR&D projects acquired from our business combinations. We performed a qualitative assessment of events and circumstances that could affect the fair value of our indefinite-lived intangible assets. Our assessment included consideration of various factors including costs associated with the underlying development programs, expected future revenues and cash flows, legal, regulatory and other entity specific factors that may have a significant impact on the inputs used to determine fair value of our indefinite-lived intangible assets. Based on our qualitative assessment, we determined that it is not more likely than not that the fair value of our indefinite-lived intangible assets is less than their carrying amounts at December 31, 2012.

At December 31, 2012, the net book value of our intangible assets whose lives are considered finite in nature was $99.3 million. These intangible assets are related to marketing rights for Naglazyme, Kuvan and Firdapse, which are being amortized over their estimated useful lives using the straight-line method. We review these intangible assets for impairment when facts or circumstances indicate a reduction in the fair value below their carrying amount.

As of December 31, 2012, we had goodwill of $51.5 million resulting from our business combinations. We currently operate in one business segment, the biopharmaceutical development and commercialization segment. When reviewing goodwill for impairment, we assess whether goodwill should be allocated to operating levels lower than our single operating segment for which discrete financial information is available and reviewed for decision-making purposes. These lower levels are referred to as reporting units. Currently, we have identified only one reporting unit as per Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 350-20, *Intangibles Goodwill and Other*. We perform our annual impairment review of goodwill during the fourth quarter and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. Our impairment review was based on a qualitative assessment including expected future revenues and cash flows, industry and market considerations and other entity specific factors that may have a significant impact on the fair value of our goodwill. Based on our qualitative assessment, we determined that it is not more likely than not that the fair value of our goodwill is less than its carrying amount at December 31, 2012.

Revenue Recognition

We recognize revenue in accordance with FASB ASC Subtopics ASC 605-15, *Revenue Recognition Products* and ASC 605-25, *Revenue Recognition Multiple-Element Arrangements.* Our revenues consist of net product revenues from commercial products, revenues from our collaborative agreement with Merck Serono and other license and royalty revenues. Milestone payments are recognized in full when the related milestone performance goal is achieved and we have no future performance obligations related to that payment.

Net Product Revenues We recognize net product revenue when persuasive evidence of an arrangement exists, the product has been delivered to the customer, title and risk of loss have passed to the customer, the price to the buyer is fixed or determinable and collection from the customer is reasonably assured. Product sales transactions are evidenced by customer purchase orders, customer contracts, invoices and/or the related shipping documents. Amounts collected from customers and remitted to governmental authorities, which are primarily comprised of value-added taxes related to Naglazyme and Firdapse sales in foreign jurisdictions, are presented on a net basis in our Consolidated Statements of Operations, in that taxes billed to customers are not included as a component of net product revenues.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

We receive a 39.5% to 50% royalty on worldwide net Aldurazyme sales by Genzyme depending on sales volume, which is included in net product revenues in the Consolidated Statements of Operations. We recognize a portion of this amount as product transfer revenue when the product is released to Genzyme because all of our performance obligations are fulfilled at that point and title to, and risk of loss for, the product has transferred to Genzyme. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay us if the product is unsold by Genzyme. The amount of product transfer revenue will eventually be deducted from the calculated royalty rate when the product is sold by Genzyme. We record the Aldurazyme royalty revenue based on net sales information provided by Genzyme and record product transfer revenue based on the fulfillment of Genzyme purchase orders in accordance with the terms of the related agreements with Genzyme and when the title and risk of loss for the product is transferred to Genzyme. As of December 31, 2012 and 2011, accounts receivable included $32.4 million and $31.0 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme.

We sell Naglazyme worldwide, Kuvan in the U.S. and Canada and Firdapse in the EU. In the U.S., Naglazyme and Kuvan are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. We also sell Kuvan to Merck Serono at a price near its manufacturing cost, and Merck Serono resells the product to end users outside the U.S., Canada and Japan. The royalty earned from Kuvan product sold by Merck Serono in the EU is included as a component of net product revenues in the period earned and approximates four percent. Outside the U.S., Naglazyme and Firdapse are sold to our authorized distributors or directly to government purchasers or hospitals, which act as the end-users. We record reserves for rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are recorded. Our reserve calculations require estimates, including estimates of customer mix, to determine which sales will be subject to rebates and the amount of such rebates. We update our estimates and assumptions each quarter and record any necessary adjustments to our reserves. We record fees paid to distributors as a reduction of revenue.

We record allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether an allowance for product returns is required, including market exclusivity of the products based on their orphan drug status, the patient population, the customers limited return rights and our experience with returns. Because of the pricing of our products, the limited number of patients and customers limited return rights, most Naglazyme, Kuvan and Firdapse customers and retailers carry a limited inventory. However, certain international customers, usually government entities, tend to purchase larger quantities of product less frequently. Although such buying patterns may result in revenue fluctuations from quarter to quarter, we have not experienced an increase in product returns and do not believe these buying patterns increase the risk of product returns. We rely on historical return rates to estimate returns for our commercial products. Genzyme s contractual return rights for Aldurazyme are limited to defective product. Based on these factors and the fact that we have not experienced significant product returns to date, management has concluded that product returns will be minimal. In the future, if any of these factors and/or the history of product returns changes, an allowance for product returns may be required.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

The nature and amount of our current estimates of the applicable revenue dilution items that are currently applied to aggregate world-wide gross sales of Naglazyme, Kuvan and Firdapse to derive net sales are described in the table below.

Revenue Dilution Item	Percentage of Gross Sales Years Ended December 31, 2012	2011	Description
Rebates	0.9-5.0%	0.9-3.2%	Rebates payable to state Medicaid, other government programs and certain managed care providers
Distributor Fees	0.3-3.8%	0.3-2.9%	Fees paid to authorized distributors
Cash Discounts	0.5-1.9%	0.5-1.9%	Discounts offered to customers for prompt payment of accounts receivable
Total	1.7-10.7%	1.7-8.0%	

We maintain a policy to record allowances for doubtful accounts for estimated losses resulting from our customers inability to make required payments. As of December 31, 2012, our allowance for doubtful accounts was $0.3 million, compared to $0.5 million as of December 31, 2011.

Royalty and license revenues Royalty and license revenues includes royalties on net sales of products with which we have no direct involvement and is recognized based on data reported by licensees or sublicensees. Royalties are recognized as earned in accordance with the contract terms when the royalty amount is fixed or determinable based on information received from the sublicensee and when collectibility is reasonably assured.

Due to the significant role we play in the operations of Aldurazyme and Kuvan, primarily the manufacturing and regulatory activities, as well as the rights and responsibilities to deliver the products to Genzyme and Merck Serono, respectively, we elected not to classify the Aldurazyme and Kuvan royalties earned as other royalty revenues and instead to include them as a component of net product revenues.

Inventory

We value our inventories at the lower of cost or net realizable value. We determine the cost of inventory using the average-cost method. We analyze our inventory levels quarterly and write down inventory that has become obsolete, or has a cost basis in excess of its expected net realizable value and inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as cost of sales on the Consolidated Statements of Operations.

Inventories consist of currently marketed products and certain products awaiting regulatory approval. In evaluating the recoverability of inventories produced in preparation for product launches, we consider the likelihood that revenue will be obtained from the future sale of the related inventory together with the status of the product within the regulatory approval process. When regulatory approval and the likelihood of future revenues for a product candidate are less certain, the related manufacturing costs expensed as research and development expenses.

Recent Accounting Pronouncements

See Note 4 to our accompanying Consolidated Financial Statements for a full description of recent accounting pronouncements and our expectation of their impact on our consolidated results of operations and financial condition.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Results of Operations

Net Income (Loss)

Our net loss for the year December 31, 2012 was $114.3 million, compared to net loss of $53.8 million for the year ended December 31, 2011. The increase in net loss was primarily a result of the following (in millions):

Net loss for the year ended December 31, 2011	$ (53.8)
Increased gross profit from product sales	51.0
Increased research and development expense	(87.8)
Increased selling, general and administrative expense	(22.7)
Increased intangible asset amortization and contingent consideration expense	(10.6)
Impairment loss on intangible assets	(6.7)
Decreased income tax expense	14.1
Loss on conversion of promissory note	(2.0)
Absence of debt conversion expense	1.9
Other individually insignificant fluctuations	2.3
Net loss for the year ended December 31, 2012	$ (114.3)

The increase in gross profit from product sales during the year ended December 31, 2012 as compared to the year ended December 31, 2011 was primarily a result of additional Naglazyme patients initiating therapy and additional Kuvan patients initiating therapy in the U.S. The increase in research and development expense was primarily attributed to increased development expenses for our Vimizim, BMN-701, BMN-673 and PEG-PAL programs. The increase in selling, general and administrative expense was primarily due to increased facility and employee related costs and the continued international expansion of Naglazyme.

Our net loss for the year ended December 31, 2011 was $53.8 million, compared to net income of $205.8 million for the year ended December 31, 2010. The change in net income (loss) was primarily a result of the following (in millions):

Net income for the year ended December 31, 2010	$ 205.8
Absence of benefit from the reversal of deferred tax asset valuation allowance	(230.6)
Increased gross profit from product sales	54.2
Increased research and development expense	(67.1)
Increased selling, general and administrative expense	(23.7)
Decreased intangible asset amortization and contingent consideration expense	5.0
Decreased debt conversion expense	11.9
Increased income tax expense, excluding valuation allowance reversal	(6.9)
Other individually insignificant fluctuations	(2.4)
Net loss for the year ended December 31, 2011	$ (53.8)

The increase in gross profit from product sales during the year ended December 31, 2011 as compared to the year ended December 31, 2010 was primarily a result of additional Naglazyme patients initiating therapy, additional Kuvan patients initiating therapy in the U.S. and increased Firdapse sales in Europe. The increase in research and development expense was primarily attributed to increased development expenses for our Vimizim,

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

PEG-PAL, Firdapse, BMN-701 and BMN-673 programs. The increase in selling, general and administrative expense was primarily due to increased facility and employee related costs, continued international expansion of Naglazyme, U.S. commercialization activities related to Kuvan, the commercialization of Firdapse in Europe and increased bad debt expense.

See below for additional information related to the primary net income/(loss) fluctuations presented above, including details of our operating expense fluctuations.

Net Product Revenues, Cost of Sales and Gross Profit

Net product revenues were as follows (in millions):

	Year Ended December 31,				
	2012	2011	2010	2012 v. 2011	2011 v. 2010
Naglazyme	$ 257.0	$ 224.9	$ 192.7	$ 32.1	$ 32.2
Kuvan	143.1	116.8	99.4	26.3	17.4
Firdapse	14.2	13.1	6.4	1.1	6.7
Aldurazyme	82.2	82.8	71.2	(0.6)	11.6
Total net product revenues	$ 496.5	$ 437.6	$ 369.7	$ 58.9	$ 67.9

Gross profit by product was as follows (in millions):

	Year Ended December 31,				
	2012	2011	2010	2012 v. 2011	2011 v 2010
Naglazyme	$ 218.5	$ 186.9	$ 158.3	$ 31.6	$ 28.6
Kuvan	118.9	98.1	82.7	20.8	15.4
Firdapse	11.4	10.8	5.0	0.6	5.8
Aldurazyme	55.8	57.8	53.4	(2.0)	4.4
Total gross profit	$ 404.6	$ 353.6	$ 299.4	$ 51.0	$ 54.2

Net product revenues attributed to our collaboration with Genzyme were as follows (in millions):

	Year Ended December 31,				
	2012	2011	2010	2012 v. 2011	2011 v. 2010
Aldurazyme revenue reported by Genzyme	$ 193.1	$ 185.2	$ 166.8	$ 7.9	$ 18.4
Royalties earned from Genzyme	$ 80.4	$ 74.2	$ 68.0	$ 6.2	$ 6.2
Incremental (previously recognized) Aldurazyme product transfer revenue	1.8	8.6	3.2	(6.8)	5.4
Total Aldurazyme net product revenues	$ 82.2	$ 82.8	$ 71.2	$ (0.6)	$ 11.6

2012 compared to 2011

Naglazyme net product revenues for the year ended December 31, 2012 totaled $257.0 million, of which $222.8 million was earned from customers based outside the U.S. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar was negative by $0.9 million for the year ended December 31, 2012. Naglazyme gross margins in 2012 were 85%, compared to 2011 when Naglazyme gross margins were 83%.The increased Naglazyme gross margins in 2012 were consistent with expectations and primarily a result of our purchase of the Naglazyme royalty rights from SA Pathology in

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November 2011 and the price increase in the U.S. and Latin America that occurred in March 2012. Prior to the purchase of the royalty rights, we licensed the intellectual property from SA Pathology to whom we paid a 5% royalty on net sales of Naglazyme. For additional discussion of the transaction see Note 6 to the Consolidated Financial Statements.

Net product revenue for Kuvan for the year ended December 31, 2012 was $143.1 million, compared to $116.8 million for the year ended December 31, 2011. Kuvan gross margins for 2012 were 83%, compared to 2011 when gross margins were 84%. Cost of goods sold for the years ended December 31, 2012 and 2011 reflect royalties paid to third-parties of 10%. Kuvan gross margins in 2012 were consistent with expectations and are not expected to fluctuate significantly in the future. The 4% royalties earned from Merck Serono s net sales of Kuvan during 2012 were $1.9 million, compared to $1.6 million during 2011.

The Hatch Waxman Act permits the FDA to approve abbreviated new drug applications, (ANDA), for generic versions of branded drugs. See Part I. Item 1 Business Government Regulation for details related to the Hatch-Waxman Act. Pursuant to the Hatch-Waxman Act, other companies were able to file an ANDA for the active ingredient in Kuvan at any time after December 2011. If a generic competitor were to enter the market following the expiration of orphan exclusivity it would have an adverse effect on our sales of Kuvan.

Net product revenue for Firdapse during the year ended December 31, 2012 was $14.2 million, compared to $13.1 million during the year ended December 31, 2011. Firdapse gross margins during 2012 were 80%, compared to the 82% during 2011. Cost of goods sold for the periods presented reflect royalties paid to third-parties of approximately 8%. Firdapse gross margins for the year ended December 31, 2012 decreased compared to the year ended December 31, 2011 due to increased manufacturing costs and the depletion of inventory manufactured prior to approval. Firdapse gross margins during 2012 were consistent with expectations and are not expected to fluctuate significantly in the future.

During the year ended December 31, 2012, Aldurazyme gross margins were 68%, compared to 70% during the year ended December 31, 2011. Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. The change in margins is attributed to the shift in revenue mix between royalty revenue and net product transfer revenues. Aldurazyme gross margins are expected to fluctuate depending on the mix of royalty revenue, from which we earn higher gross profit, and product transfer revenue, from which we earn lower gross profit.

Total cost of sales for the year ended December 31, 2012 was $91.8 million, compared to $84.0 million for the year ended December 31, 2011. The increase in cost of sales was primarily attributed to the increase in product sales and the amortization of the cost of the Naglazyme royalty rights purchased in the fourth quarter of 2011 and the shift in Aldurazyme revenue mix between royalty revenue and net product revenues.

2011 compared to 2010

Naglazyme net product revenues for the year ended December 31, 2011 totaled $224.9 million, of which $194.2 million was earned from customers based outside the U.S. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar was negative by $0.2 million for 2011. Gross margins from Naglazyme sales during 2011 were 83%, compared to 82% during 2010. Naglazyme gross margins for the year ended December 31, 2011 were consistent with expectations and were expected to improve slightly in 2012 as a result of our purchase of the Naglazyme intellectual property from SA Pathology in November 2011. Prior to the purchase, we licensed the intellectual property from SA Pathology and paid them a 5% royalty on net sales of Naglazyme. See Note 6 to our accompanying Consolidated Financial Statements for additional discussion of the transaction.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Net product revenue for Kuvan for the year ended December 31, 2011 was $116.8 million, compared to $99.4 million for the year ended December 31, 2010. Gross margins from Kuvan during 2011 were approximately 84%, compared to 83% during 2010. The increase in gross margins was primarily attributed to price increases at the end of 2010. Cost of goods sold for the years ended December 31, 2011 and 2010 reflect royalties paid to third parties of 10% and 11%, respectively. The 4% royalties from Merck Serono s net sales of Kuvan during 2011 were $1.6 million, compared to $0.9 million in 2010. Kuvan gross margins for the year ended December 31, 2011 were consistent with expectations.

We launched Firdapse in Europe on a country-by-country basis beginning in April 2010. Net product revenue for Firdapse for the year ended December 31, 2011 was $13.1 million, compared to $6.4 million for the year ended December 31, 2010. Gross margins from Firdapse during 2011 were 82% compared to 79% during 2010. Cost of goods sold for the years ended December 31, 2011 and 2010 reflect royalties paid to third parties of approximately 8%.

During the year ended December 31, 2011, Aldurazyme gross margins were 70%, compared to the year ended December 31, 2010 when gross margins were 75%. Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. The change in margins is attributed to the shift in revenue mix between royalty revenue and net product transfer revenues. Aldurazyme gross margins are expected to fluctuate depending on the mix of royalty revenue, from which we earn higher gross profit, and product transfer revenue, from which we earn lower gross profit.

Total cost of sales for the year ended December 31, 2011 was $84.0 million, compared to $70.3 million for the year ended December 31, 2010. The increase in cost of sales during 2011 compared to 2010 was primarily attributed to the increase in product sales and the shift in Aldurazyme revenue mix between royalty revenue and net product revenues.

Research and Development

We manage our Research and development expense by identifying the research and development activities we anticipate will be perform during a given period and then prioritize efforts based on scientific data, probability of successful development, market potential, available human and capital resources and other similar considerations. We continually review our pipeline and the development status of product candidates, and as necessary, reallocate resources among the research and development portfolio that we believe will best support the future growth of our business.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Research and development expense increased to $302.2 million for the year ended December 31, 2012, from $214.4 million for the year ended December 31, 2011. The increase in research and development expense was primarily a result of the following (in millions):

Research and development expense for the year ended December 31, 2011	$ 214.4
Increased Vimizim development expenses	42.5
Increased BMN-701 development expenses	14.1
Increased BMN-190 development expenses	9.9
Increased BMN-673 development expenses	4.0
Increased stock-based compensation expense related to research and development	4.4
Decreased development expense related to commercial products	(1.6)
Decreased BMN-111 development expenses	(1.5)
Decreased PEG-PAL development expenses	(1.0)
Decreased development expenses on early development stage programs	(0.7)
Increase in non-allocated research and development expenses and other net changes	17.7
Research and development expense for the year ended December 31, 2012	$ 302.2

The increase in Vimizim development expenses was attributed to increased clinical trial and manufacturing activities related to the product candidate. The increase in BMN-673 and BMN-701 development expenses were attributed to increased clinical trial activities related to these product candidates. The increase in BMN-190 development expense was attributed to increased pre-clinical activities related to this product candidate. The decrease in PEG-PAL development expense was attributed to the timing of purchases of materials to produce the drug substance for the clinical trial. The decrease in BMN-111 development expense was attributed to a decrease in pre-clinical activities related to this product candidate. The increase in stock-based compensation expense is a result of an increased number of options outstanding due to an increased number of employees. The increase in non-allocated research and development expense primarily includes increased research and development personnel and facility costs that are not allocated to specific programs.

In 2013, we expect research and development spending to increase over 2012 levels due to our Vimizim, PEG-PAL, BMN-673, BMN-701, BMN-111 and BMN-190 programs progressing to more advanced phases of clinical studies as well as increased spending on pre-clinical and clinical activities for our early development stage programs. Additionally, we expect to continue incurring significant research and development expense for the foreseeable future due to long-term clinical activities related to post-approval regulatory commitments for our approved products. We continuously evaluate the recoverability of costs associated with prelaunch manufacturing activities, and if it is determined that regulatory approval and recoverability are highly likely and therefore future revenues are expected, the costs related to prelaunch manufacturing activities may be capitalized. When regulatory approval and the likelihood of future revenues for a product candidate are less certain, the related manufacturing costs expensed as research and development expenses.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Research and development expense increased to $214.4 million for the year ended December 31, 2011, from $147.3 million for the year ended December 31, 2010. The increase in research and development expense was primarily a result of the following (in millions):

Research and development expense for the year ended December 31, 2010	$ 147.3
Increased Vimizim development expenses	26.4
Increased BMN-701 development expenses	15.0
Increased PEG-PAL development expenses	11.3
Increased BMN-111 development expenses	11.3
Increased ongoing development expenses related to commercial products	2.8
Increased stock-based compensation expense related to research and development	2.6
Decreased BMN-195 for Duchenne muscular dystrophy development expenses	(3.3)
Decreased BMN-673 development expenses	(0.9)
Increase in non-allocated research and development expenses and other net changes	1.9
Research and development expense for the year ended December 31, 2011	$ 214.4

The increase in Vimizim, PEG-PAL, BMN-673 and BMN-701 development expenses were attributed to increased clinical trial activities related to these product candidates. The increase in research and development expenses related to commercial products was primarily attributed to long-term Firdapse clinical activities related to post-approval regulatory commitments in the EU. The decrease in development expense related to BMN-195 was attributed to the termination of our license agreement with Summit plc in October 2010. The increase in stock-based compensation expense was a result of an increased number of options outstanding due to an increased number of employees. The increase in non-allocated research and development expense primarily included increased research and development personnel costs that were not allocated to specific programs.

Selling, General and Administrative

Selling, general and administrative expense increased to $198.2 million for the year ended December 31, 2012, from $175.4 million for the year ended December 31, 2011. The increase in selling, general and administrative expenses was primarily a result of the following (in millions):

Selling, general and administrative expense for the year ended December 31, 2011	$ 175.4
Net increase in corporate support and other administrative expenses	16.0
Increased sales and marketing expenses related to commercial products	6.2
Increased Vimizim pre-commercial expenses	2.9
Increased foreign exchange losses on unhedged transactions	(2.3)
Selling, general and administrative expense for the year ended December 31, 2012	$ 198.2

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

The increase in corporate support and other administrative costs was primarily comprised of increased employee-related costs and facility costs. The increase in employee-related costs was primarily attributed to the increase in headcount. The increase in facility costs was primarily driven by the occupation of our new corporate headquarters in San Rafael, California. We continue to incur sales and marketing expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. We expect selling, general and administrative expenses to increase in future periods as a result of the international expansion of Naglazyme, the U.S. commercialization activities for Kuvan and the administrative support of our expanding operations.

Selling, general and administrative expense increased to $175.4 million for the year ended December 31, 2011, from $151.7 million for the year ended December 31, 2010. The increase in selling, general and administrative expenses was primarily a result of the following (in millions):

Selling, general and administrative expense for the year ended December 31, 2010	$ 151.7
Net increase in corporate overhead and other administrative expenses	12.4
Increased sales and marketing expenses related to commercial products	8.4
Increased foreign exchange loss on unhedged transactions	1.9
Increased Vimizim pre-commercial expense	1.7
Increased bad debt expense	1.1
Absence of transaction costs related to the acquisition of ZyStor Therapeutics, Inc (ZyStor)	(1.8)
Selling, general and administrative expense for the year ended December 31, 2011	$ 175.4

We continue to incur sales and marketing expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively, and spending related to the European commercialization of Firdapse, which launched in the EU in April 2010. The increase in corporate overhead and other administrative costs during 2011 was primarily comprised of increased employee related costs, legal costs, accounting costs and facility costs.

Intangible Asset Amortization and Contingent Consideration

Intangible asset amortization and contingent consideration expense is comprised of amortization of the European marketing rights for Firdapse, changes in the fair value of contingent acquisition consideration payable to former stockholders of our acquired businesses and impairment loss (if any) on intangible assets. Changes in the fair value of contingent acquisition consideration payable result from updates to the assumed probability of achievement or timing of milestones and adjustments to the discount periods and rates. Intangible asset amortization and contingent consideration expense consisted of the following (in millions):

	Year Ended December 31,			Change	
	2012	2011	2010	2012 v. 2011	2011 v 2010
Amortization of Firdapse European marketing rights	$ 3.2	$ 3.2	$ 2.4	$ 0	$ 0.8
Impairment loss on intangible assets	6.7	0	0	6.7	0
Changes in the fair value of contingent acquisition consideration payable	8.8	(1.8)	4.0	10.6	(5.8)
Total intangible asset amortization and contingent consideration	$ 18.7	$ 1.4	$ 6.4	$ 17.3	$ (5.0)

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

In the first quarter of 2012, we recorded an impairment charge of $6.7 million related to the U.S. Firdapse in-process research and development (IPR&D) assets based on the status of business development efforts at the time and the related discounted cash flows that no longer supported the carrying-value of the IPR&D assets. The IPR&D assets impaired were associated with the marketing rights for Firdapse in the U.S. The increase in the fair value of the contingent acquisition consideration payable was primarily attributed to increases in the assumed probability of achieving development milestones based on the current status of the related development programs.

The 2011 increase in the amortization of the Firdapse European marketing rights was attributed to the timing of the European commercial launch of Firdapse which occurred in April 2010.

See Note 6 to our accompanying Consolidated Financial Statements for additional discussion.

Equity in the Loss of BioMarin/Genzyme LLC

Equity in the loss of BioMarin/Genzyme LLC includes our 50% share of the joint venture s loss for the period. BioMarin/Genzyme LLC s operations consist primarily of certain research and development activities and the intellectual property that are managed by the joint venture, with costs shared equally by BioMarin and Genzyme.

Equity in the loss of the joint venture totaled $1.2 million for the year ended December 31, 2012 compared to $2.4 million and $3.0 million for the years ended December 31, 2011 and 2010, respectively.

Interest Income

We invest our cash, short-term and long-term investments in government and other high credit quality securities in order to limit default and market risk. Interest income totaled $2.6 million for the year ended December 31, 2012, compared to $2.9 million and $4.1 million for the years ended December 31, 2011 and 2010, respectively. The reduction in interest income during 2012, as compared to 2011 and 2010 was primarily due to lower market interest rates. We expect that interest income will increase during 2013 as compared to 2012 due to higher cash and investment balances resulting from the net proceeds received from the sale of 6.5 million shares of our common stock in June 2012.

Interest Expense and Debt Conversion Expense

We incur interest expense on our convertible debt and our capital leases. Interest expense for the year ended December 31, 2012 was $7.6 million, compared to $8.4 million and $10.8 million for the years ended December 31, 2011 and 2010, respectively. The decrease in interest expense was attributed to the early conversion of $29.2 million and $119.6 million in aggregate principal of our 2013 Notes in September 2011 and November 2010, respectively. In connection with the early conversion of the 2013 Notes, we recognized debt conversion expense of $1.9 million and $13.7 million in 2011 and 2010, respectively. We expect interest expense to decrease in 2013, compared to 2012 as our senior subordinated convertible notes mature in March 2013. See Note 11 to our accompanying Consolidated Financial Statements for additional discussion.

Provision for (Benefit from) Income Taxes

During the year ended December 31, 2012 we recognized an income tax benefit of $3.9 million, compared to an income tax expense of $10.2 million and an income tax benefit of $227.3 million for the years ended December 31, 2011 and 2010, respectively. The provision for (benefit from) income taxes for 2012 and 2011 consisted of state, federal and foreign current tax expense of $6.0 million and $5.8 million, respectively. The provision for (benefit from) income taxes also consisted of deferred tax expense related to the utilization of our

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federal net operating loss carryforwards and a portion of our credit carryforwards which were offset by deferred tax benefits from federal orphan drug credits and California R&D credits of $32.6 million and $19.2 million earned during 2012 and 2011, respectively. The benefit from income tax during 2010 consisted of foreign and state current tax expense and deferred tax benefit related to the release of $230.6 million of our valuation allowance during 2010. See Note 19 to our accompanying Consolidated Financial Statements for additional discussion of the components of our provision for (benefit from) income taxes.

The consolidated U.S. GAAP loss includes all of our foreign subsidiaries. In accordance with ASC 740, *Income Taxes,* we calculate our provision for (benefit from) income taxes on an entity-by-entity and jurisdiction-by-jurisdiction basis as adjusted for differences between book-basis income and tax-basis income, which results in certain foreign entities being profitable and incurring foreign current income tax expense. Certain foreign entities incur significant amounts of research and development expense that results in significant losses that more than offset the income reported by the profitable foreign entities on a consolidated basis. The majority of these material research and development losses are in foreign jurisdictions that do not have net operating loss carryforward provisions that result in deferred tax assets, which results in an effective tax rate of 0% on approximately $168.0 million of foreign net losses. Other foreign operations generated U.S. GAAP income of approximately $5.0 million with an effective tax rate of approximately 38%.

Financial Position, Liquidity and Capital Resources

We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents, short-term and long-term investments supplemented by proceeds from equity or debt financings and loans or collaborative agreements with corporate partners, each to the extent necessary. We expect our current cash, cash equivalents and short-term and long-term investments will meet our operating and capital requirements for at least the next twelve months based on our current business plans. This expectation could also change depending on how much we elect to spend on our development programs and for potential licenses and acquisitions of complementary technologies, products and companies. In June 2012, we sold 6.5 million shares of our common stock at a price of $36.28 per share in an underwritten public offering pursuant to an effective registration statement. We received net cash proceeds of $235.5 million from the public offering. We will be highly dependent on our net product revenue to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be invested indefinitely outside the U.S. As of December 31, 2012, $17.1 million of our $566.7 million balance of cash, cash equivalents, and marketable securities was from foreign subsidiary operations and is intended to fund future foreign operations. In managing our liquidity needs in the U.S., we do not rely on the unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings.

We are mindful that conditions in the current macroeconomic environment could affect our ability to achieve our goals. Some of the factors that could affect our business include: future changes to healthcare reform in the U.S., a continuation or worsening of global economic conditions, patent expirations of competitive products and the launch of generic competitors, continued government pricing pressures internationally and the potential volatility in foreign currency exchange rates. We will continue to monitor these conditions and will adjust our business processes, as appropriate, to mitigate these risks to our business.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Our financial condition as of each year ended December 31 was as follows (in millions):

	2012	2011	2010	2012 v. 2011	2011 v. 2010
Cash and cash equivalents	$ 180.5	$ 46.3	$ 88.1	$ 134.2	$ (41.8)
Short-term investments	270.2	148.8	186.0	121.4	(37.2)
Long-term investments	116.0	94.4	128.2	21.6	(33.8)
Cash, cash equivalents and investments	$ 566.7	$ 289.5	$ 402.3	$ 277.2	$ (112.8)
Current assets	$ 743.5	$ 469.8	$ 504.3	$ 273.7	(34.5)
Current liabilities	170.4	94.1	83.8	76.3	10.3
Working capital	$ 573.1	$ 375.7	$ 420.5	$ 197.4	$ (44.8)
Convertible debt	$ 348.2	$ 348.3	$ 377.5	$ (0.1)	$ (29.2)

Our cash flows for each of the years ended December 31 is summarized as follows (in millions):

	2012	2011	2010	2012 v. 2011	2011 v. 2010
Cash and cash equivalents at the beginning of the period	$ 46.3	$ 88.1	$ 167.2	$ (41.8)	$ (79.1)
Net cash provided by operating activities	17.6	18.8	18.7	(1.2)	0.1
Net cash used in investing activities	(195.6)	(89.6)	(101.3)	(106.0)	11.7
Net cash provided by financing activities	312.2	29.0	3.5	283.2	25.5
Cash and cash equivalents at the end of the period	$ 180.5	$ 46.3	$ 88.1	$ 134.2	$ (41.8)
Short-term and long-term investments	386.2	243.2	314.2	143.0	(71.0)
Cash, cash equivalents and investments	$ 566.7	$ 289.5	$ 402.3	$ 277.2	$ (112.8)

Cash, cash equivalents and investments

The increase in cash, cash equivalents and investments in 2012 from December 31, 2011 was primarily attributed to the net proceeds of $235.5 million from our public offering of our common stock in June 2012, proceeds from employee stock purchases under the Employee Stock Purchase Plan (ESPP) and employee stock option exercises of $81.4 million.

The decrease in cash, cash equivalents and investments in 2011 from December 31, 2010 was primarily attributed to the $49.7 million of cash used in the purchase of the Shanbally facility and the $81.0 million purchase of the Naglazyme intellectual property (Naglazyme IP), partially offset by proceeds from employee stock purchases under the ESPP and employee stock option exercises of $33.6 million.

Working Capital

Working capital was $573.1 million at December 31, 2012, an increase of $197.4 million from working capital of $375.7 million at December 31, 2011. The increase in working capital was attributed to the following:

Working capital at December 31, 2011	$ 375.7

Increased cash, cash equivalents and short-term investments	255.6
Increased current deferred tax assets	8.3
Increased accounts payable and accrued liabilities	(52.9)
Reclassification of 2013 Notes from long-term convertible debt	(23.4)
Net increase in other current operating assets	9.8
Working capital at December 31, 2012	$ 573.1

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

The increase in cash, cash equivalents and short-term investments resulted from net proceeds of $235.5 million from the public offering of our common stock in June 2012. The increase in other current assets is primarily attributed to the increases in prepaid expenses, current deferred tax assets and short-term restricted investments. The restricted investments secure our irrevocable standby letter of credit obtained in connection with our new corporate facility lease agreements and certain other commercial arrangements. The classification of the 2013 Notes as a current liability from long-term convertible debt was due to their maturity in March 2013.

Our product sales to government-owned or government-funded customers in certain Southern European countries, including Greece, Spain, Italy and Portugal are subject to payment terms that are imposed by government authority. Because these customers are government-owned or government-funded, we may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in Greece, or in other Southern European countries, may decrease the likelihood that we will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to our operating results. Historically we have not experienced a significant level of uncollected receivables and have received continued payments from our more aged accounts. We believe that the allowances for doubtful accounts for these countries are adequate based on our analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. As of December 31, 2012, approximately 8% of our outstanding accounts receivable relate to such countries. See Note 18 of our accompanying Consolidated Financial Statements for additional discussion.

Cash Provided by Operating Activities

Cash provided by operating activities for the year ended December 31, 2012 was $17.6 million, compared to cash provided by operating activities of $18.8 million for the year ended December 31, 2011. The decrease in cash provided by operating activities was primarily related to increased research and development expense that drove the increase in our net loss of $114.3 million, adjusted for non-cash items such as $45.3 million of depreciation and amortization expenses, $47.3 million of stock-based compensation expense, $6.7 million of impairment loss on intangible assets, $8.8 million decrease in the fair value of contingent acquisition consideration payable, $9.9 million decrease in deferred income taxes, $6.5 million of unrealized foreign exchange gain on forward foreign currency exchange contracts and $33.1 million of net cash inflow related to changes in operating assets and liabilities.

Cash provided by operating activities of $18.8 million for the year ended December 31, 2011 primarily related to net loss of $53.8 million, adjusted for non-cash items such as $36.1 million of depreciation and amortization expenses, $43.9 million of stock-based compensation expense, $4.4 million of deferred income taxes and $7.2 million of unrealized foreign exchange gains on forward foreign currency exchange contracts and $25.1 million of net cash outflows related to changes in operating assets and liabilities.

Cash Used in Investing Activities

Net cash used in investing activities during the year ended December 31, 2012 was $195.6 million, compared to net cash used in investing activities of $89.6 million and $101.3 million for the years ended December 31, 2011 and 2010, respectively. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. The increase in net cash used in investing for the year ended December 31, 2012 was primarily comprised of a $210.9 million increase in net purchases of available-for-sale investments, offset by a $81.0 million decrease in purchases of intellectual property and a $28.6 million decrease in capital expenditures. The decrease in net cash used in investing activities for the year

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ended December 31, 2011 compared to the year ended December 31, 2010 was primarily due to increased capital expenditures of $23.8 million and lower spending on business acquisitions of $33.0 million, partially offset by the $81.0 million purchase of Naglazyme IP and increased net settlements of investment securities of $81.9 million. In 2011, capital expenditures were primarily comprised of our purchase of the Shanbally facility for a total purchase price of $49.7 million.

Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2012 was $312.2 million, compared to net cash provided by financing activities of $29.0 million and $3.5 million for the years ended December 31, 2011 and 2010, respectively. Historically, our financing activities primarily included payments related to our contingent acquisition obligations, payments related to our convertible debt obligations and proceeds from employee stock purchases under the ESPP and employee stock option exercises. The increase in net cash provided by financing activities for the year ended December 31 2012, was primarily attributed to the June 2012 public offering of our common stock which generated net cash proceeds of $235.5 million, an increase of $47.8 million in proceeds from the ESPP and employee stock option exercises, a $2.2 million decrease in debt conversion expense, offset by a $2.5 million decrease in payments of contingent acquisition consideration. The increase in net cash provided by financing activities for the year ended December 31, 2011, was primarily attributed to the decrease in payments of contingent acquisition consideration of $14.0 million and lower induced debt conversion payments of $11.9 million. See Notes 11 and 13 to our accompanying Consolidated Financial Statements for additional discussion regarding our convertible debt and the June 2012 public offering of our common stock, respectively.

Other Information

In March 2006, we sold approximately $172.5 million of senior subordinated convertible notes due March 2013 (the 2013 Notes) of which $23.4 million remains outstanding at December 31, 2012. The debt was issued at face value and bears interest at the rate of 2.5% per annum, payable semi-annually in cash. The debt does not contain a call provision included and we are unable to unilaterally redeem the remaining debt prior to maturity in 2013. The remaining $23.4 million of the 2013 Notes is convertible, at the option of the holder, at any time prior to maturity, into shares of our common stock at a conversion price of approximately $16.58 per share, subject to adjustment in certain circumstances. However, we must repay the remaining debt prior to maturity if there is a qualifying change in control or termination of trading of our common stock. If a change of control occurs, we will pay a make whole premium by increasing the conversion rate applicable to the notes.

In April 2007, we sold approximately $324.9 million of senior subordinated convertible notes due April 2017 (the 2017 Notes). The debt was issued at face value and bears interest at the rate of 1.875% per annum, payable semi-annually in cash. The debt is convertible, at the option of the holder, at any time prior to maturity, into shares of our common stock at a conversion price of approximately $20.36 per share, subject to adjustment in certain circumstances. Our debt does not contain a call provision and we are unable to unilaterally redeem the debt prior to maturity in 2017. We also must repay the debt if there is a qualifying change in control or termination of trading of our common stock. If a change of control occurs, we will pay a make whole premium by increasing the conversion rate applicable to the notes. See Note 11 to our accompanying Consolidated Financial Statements for additional discussion.

Our $348.2 million of total convertible debt as of December 31, 2012 will impact our liquidity due to the semi-annual cash interest payments and will impact our liquidity if the holders do not convert on or prior to the scheduled repayments of the debt. Further, depending on market conditions, our financial position and performance and other factors, we may in the future choose to use a portion of our cash or cash equivalents to repurchase our convertible debt or other securities.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

On October 23, 2009, we acquired Huxley Pharmaceuticals Inc. (Huxley), which has rights to Firdapse for a total purchase price of $37.2 million, of which $15.0 million was paid in cash and $22.2 million represented the acquisition date fair value of contingent acquisition consideration payable. In connection with the acquisition, we agreed to pay the Huxley stockholders additional consideration in future periods of up to $41.9 million (undiscounted) in milestone payments if certain annual sales, cumulative sales and U.S. development milestones are met. During 2011, 2010 and 2009 we made milestone payments of $3.0 million, $6.5 million and $1.0 million, respectively, related to the attainment of development milestones.

On February 10, 2010, we acquired LEAD Therapeutics, Inc. (LEAD), which had the key compound now referred to as BMN-673, for a total purchase price of $39.1 million, of which $18.6 million was paid in cash and $20.5 million represented the acquisition date fair value of contingent acquisition consideration payable. We paid $3.0 million of the $18.6 million in cash during December 2009. In connection with the acquisition, we agreed to pay the LEAD stockholders additional consideration in future periods of up to $68.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met. During 2012 and 2010, we paid the former LEAD stockholders $6.0 million and $11.0 million for the attainment of a clinical milestone and regulatory milestone, respectively.

On August 17, 2010, we acquired ZyStor, which had the compound now referred to as BMN-701, for a total purchase price of $35.9 million, of which $20.3 million was paid in cash and $15.6 million represented the acquisition date fair value of contingent acquisition consideration payable. In connection with the acquisition, we agreed to pay ZyStor stockholders additional consideration in future periods of up to $93.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met.

On January 4, 2013, we acquired Zacharon Pharmaceuticals, Inc. (Zacharon), which focused on developing small molecules targeting pathways of glycan and glycolipid metabolism, for an upfront payment of $10.0 million, of which $1.7 million was held in escrow. In connection with the acquisition, we agreed to pay the Zacharon stockholders additional consideration in future periods of up to $134.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met.

Funding Commitments

We cannot estimate with certainty the cost to complete any of our product development programs. Additionally, except as disclosed under *Overview* above, we cannot precisely estimate the time to complete any of our product development programs or when we expect to receive net cash inflows from any of our product development programs. Please see *Risk Factors* included in this Annual Report on Form 10-K for a discussion of the reasons we are unable to estimate such information, and in particular the following risk factors included in this Annual Report on Form 10-K:

if we fail to obtain or maintain regulatory approval to commercially market and sell our drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased;

if we are unable to successfully develop and maintain manufacturing processes for our drug products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program;

if we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected; and

if we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may

decline.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our research and development expenses during the three years ended December 31, 2012, 2011 and 2010 and the period since inception (March 1997 for the portion not allocated to any major program) were as follows (in millions):

	Year Ended December 31,			Since Program Inception
	2012	2011	2010	
Vimizim	$ 97.0	$ 54.5	$ 28.1	$ 211.8
Naglazyme	12.4	10.3	9.7	164.8
Kuvan	14.1	12.6	12.8	140.8
Firdapse	5.4	11.0	8.8	25.7
BMN-673	11.4	7.4	8.3	27.1
BMN-701	31.6	17.5	2.5	51.6
BMN-111	12.1	13.6	2.3	31.9
BMN-190	11.1	1.2	2.4	17.7
PEG-PAL	26.7	27.7	16.4	113.2
Not allocated to specific major current projects	80.4	58.6	56.0	Not meaningful
Totals	$ 302.2	$ 214.4	$ 147.3	

We may elect to increase our spending above our current long-term plans and consequently we may be unable to achieve our long-term goals. This may increase our capital requirements, including: costs associated with the commercialization of our products; additional clinical trials; investments in the manufacturing of Naglazyme, Aldurazyme, Kuvan and Firdapse; preclinical studies and clinical trials for our other product candidates; potential licenses and other acquisitions of complementary technologies, products and companies; and general corporate purposes.

Our future capital requirements will depend on many factors, including, but not limited to:

- our ability to successfully market and sell Naglazyme, Kuvan and Firdapse;
- Genzyme s ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;

the progress of research programs carried out by us;

our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD, ZyStor, Huxley and Zacharon that trigger related milestone payments;

any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and

whether our convertible debt is converted to common stock in the future.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements that are currently material or reasonably likely to be material to our consolidated financial position or results of operations.

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Management s Discussion and Analysis of Financial Condition and Results of Operations (Continued)

Contractual and Commercial Obligations

We have contractual and commercial obligations under our debt, operating leases and other obligations related to research and development activities, purchase commitments, licenses and sales royalties with annual minimums. Information about these obligations as of December 31, 2012 is presented in the table below (in millions).

	Payments Due by Period					
	2013	2014	2015-2016	2017-2018	2019 and Thereafter	Total
Convertible debt and related interest	$ 29,749	$ 6,091	$ 12,182	$ 327,905	$ 0	$ 375,927
Operating leases	9,015	6,749	13,486	11,582	16,424	57,256
Research and development and purchase commitments	6,378	2,992	2,620	0	0	11,990
Total	$ 45,142	$ 15,832	$ 28,288	$ 339,487	$ 16,424	$ 445,173

We are also subject to contingent payments related to various development activities totaling approximately $285.5 million as of December 31, 2012, which are due upon achievement of certain development and commercial milestones, and if they occur before certain dates in the future. As of December 31, 2012 $41.4 million of the contingent payments are included in contingent acquisition consideration payable on our accompanying Consolidated Balance Sheets, of which $10.8 million is current.

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Item 7A. Quantitative and Qualitative Disclosure About Market Risk

Interest Rate Market Risk

Our exposure to market risk for changes in interest rates relates primarily to our investment portfolio. By policy, we place our investments with highly rated credit issuers and limit the amount of credit exposure to any one issuer. As stated in our investment policy, we seek to improve the safety and likelihood of preservation of our invested funds by limiting default risk and market risk.

We mitigate default risk by investing in high credit quality securities and by positioning our portfolio to respond appropriately to a significant reduction in a credit rating of any investment issuer or guarantor. The portfolio includes only marketable securities with active secondary or resale markets to ensure portfolio liquidity.

As of December 31, 2012, our investment portfolio did not include any investments with significant exposure to the subprime mortgage market issues or the European debt crisis. Based on our investment portfolio and interest rates at December 31, 2012, we believe that a 100 basis point decrease in interest rates could result in a potential loss in fair value of our investment portfolio of approximately $5.0 million. Changes in interest rates may affect the fair value of our investment portfolio. However, we will not recognize such gains or losses in our Consolidated Statement of Operations unless the investments are sold.

The table below presents the carrying value of our cash and investment portfolio, which approximates fair value at December 31, 2012 (in millions):

	Carrying Value
Cash and cash equivalents	$ 180.5*
Short-term investments	270.2**
Long-term investments	116.0***
Total	$ 566.7

* 70% of cash and cash equivalents are invested in money market instruments and 30% in cash.

** 82% of short-term investments are invested in corporate debt securities, 14% in certificates of deposit, 3% in U.S. government agency securities and 1% in corporate equity securities.

*** 82% of long-term investments are invested in corporate debt securities, 10% in certificates of deposit and 8% in U.S. government agency securities.

Our debt obligations consist of our convertible debt, which carries a fixed interest rate and, as a result, we are not exposed to interest rate market risk on our convertible debt. The carrying value of our convertible debt approximates its fair value at December 31, 2012.

Foreign Currency Exchange Rate Risk

We transact business in various foreign currencies, primarily in Euros and Brazilian Real. Accordingly, we are subject to exposure from movements in foreign currency exchange rates of the Euro from sales of our products in Europe. Our operating expenses in the United Kingdom, other European countries and Brazil are in British Pounds, Euros and Real, respectively, which serve to mitigate a portion of the exposure related to the above-mentioned revenue in both markets.

We hedge a portion of our net position in assets and liabilities denominated in Euros using forward foreign currency exchange contracts. We also hedge a percentage of our forecasted Euro denominated revenue and operating expenses denominated in Brazilian Reais with forward foreign currency exchange contracts. Our hedging policy is designed to reduce the impact of foreign currency exchange rate movements. We mitigate

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short-term foreign currency exposure resulting from currency fluctuations by entering into forward foreign currency exchange contracts. These contracts have maturities of less than 17 months.

As of December 31, 2012, we had forward foreign currency exchange contracts to sell approximately 83.0 million Euros and to buy approximately 6.0 million Brazilian Reais. As of December 31, 2012, our outstanding forward foreign currency exchange contracts had a net fair value of $0.1 million, of which $1.5 million was included in other current assets, $1.0 million was included in accounts payable and accrued expenses and $0.4 million was included in other long-term liabilities on our accompanying Consolidated Balance Sheets.

We do not use derivative financial instruments for speculative trading purposes, nor do we hedge foreign currency exchange rate exposure in a manner that entirely offsets the effects of changes in foreign currency exchange rates. The counterparties to these forward foreign currency exchange contracts are creditworthy multinational commercial banks, which minimizes the risk of counterparty nonperformance. We currently do not use financial instruments to hedge operating expenses denominated in local currencies in Europe. Instead, we believe that a natural hedge exists, in that local currency revenue substantially offsets the local currency operating expenses. We regularly review our hedging program and may, as part of this review, make changes to the program.

Based on our overall foreign currency exchange rate exposures at December 31, 2012, we believe that a near-term 10% fluctuation of the U.S. dollar exchange rate could result in a potential change in the fair value of our foreign currency sensitive assets and investments by approximately $11.4 million. We expect to enter into new transactions based in foreign currencies that could be impacted by changes in exchange rates.

At December 31, 2012, we had cash of approximately $25.4 million denominated in foreign currencies, which represented approximately 14% of the total cash and investment portfolio. As a result, our cash and investment portfolio is subject to limited amounts of foreign currency exchange rate risk.

Item 8. Financial Statements and Supplementary Data

The information required to be filed in this item appears on pages F-1 to F-46 of this report.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

An evaluation was carried out, under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act as of the end of the period covered by this report. Based on the evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures are effective to ensure that the information required to be disclosed by us in the reports we file or submit under the Exchange Act was recorded, processed, summarized and reported within the time periods specified in the SEC s rules and forms.

Management s Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining an adequate internal control structure and procedures for financial reporting. Under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, our management has assessed the effectiveness of our internal control over financial reporting as defined in Rule 13a-15(f) under the Exchange Act as of December 31, 2012. Our management s assessment was based on criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission, or COSO, Internal Control-Integrated Framework.

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Based on the COSO criteria, we believe our internal control over financial reporting as of December 31, 2012 was effective.

Our independent registered public accounting firm, KPMG LLP, has audited the financial statements included in this Annual Report on Form 10-K and has issued a report on the effectiveness of our internal control over financial reporting. The report of KPMG LLP is incorporated by reference from Item 8 of this Annual Report on Form 10-K.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting, as defined in Rule 13a-15(f) under the Exchange Act.

Scope of the Effectiveness of Controls

Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Our internal control over financial reporting includes those policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect our transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and our board of directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on our financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions or that the degree of compliance with the policies or procedures may deteriorate.

Item 9B. Other Information

None

Part III

Item 10. Directors, Executive Officers and Corporate Governance

We incorporate information regarding our directors, executive officers and corporate governance into this section by reference from sections captioned Election of Directors and Executive Officers in the proxy statement for our 2013 annual meeting of stockholders.

Item 11. Executive Compensation

We incorporate information regarding executive compensation into this section by reference from the section captioned Executive Compensation in the proxy statement for our 2013 annual meeting of stockholders.

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Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

We incorporate information regarding security ownership of our beneficial owners, management and related stockholder matters into this section by reference from the section captioned Security Ownership of Certain Beneficial Owners in the proxy statement for our 2013 annual meeting of stockholders. We incorporate information regarding the securities authorized for issuance under our equity compensation plans into this section by reference from the section captioned Equity Compensation Plan Information in the proxy statement for our 2013 annual meeting of stockholders.

Item 13. Certain Relationships and Related Transactions and Director Independence

We incorporate information regarding certain relationships, related transactions and director independence into this section by reference from the section captioned Transactions with Related Persons, Promoters and Certain Control Persons in the proxy statement for our 2013 annual meeting of stockholders.

Item 14. Principal Accounting Fees and Services

We incorporate information regarding our principal accountant fees and services into this section by reference from the section captioned Independent Registered Public Accounting Firm in the proxy statement for our 2013 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

Financial Statements

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Reports of Independent Registered Public Accounting Firm	F-1
Consolidated Financial Statements as of December 31, 2012 and 2011 and for the three years ended December 31, 2012:	
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Consolidated Statements of Operations	F-4
Consolidated Statements of Comprehensive Income (Loss)	F-5
Consolidated Statements of Changes in Stockholders Equity	F-6
Consolidated Statements of Cash Flows	F-7
Notes to Consolidated Financial Statements	F-8

In accordance with Rule 3-09 of Regulation S-X, the comparative unaudited 2012 and 2011 and audited 2010 Consolidated Financial Statements and accompanying notes of BioMarin/Genzyme LLC, which constituted a significant subsidiary in 2010 are filed herewith as Exhibit 99.1 to this Annual Report on Form 10-K.

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Exhibit Index

3.1	Amended and Restated Certificate of Incorporation, as amended June 12, 2003, previously filed with the Commission on June 23, 2003 as Exhibit 3.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
3.2	Certificate of Correction to Certificate of Amendment to the Amended and Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., dated April 4, 2005, previously filed with the Commission on April 5, 2005 as Exhibit 3.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
3.3	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc. as filed with the Delaware Secretary of State on October 12, 2007, previously filed with the Commission on February 22, 2012 as Exhibit 3.3 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
3.4	Amended and Restated By-Laws of BioMarin Pharmaceutical Inc., previously filed with the Commission on December 23, 2010 as Exhibit 3.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
3.5	Certificate of Elimination of Series B Junior Participating Preferred Stock, dated May 30, 2012, previously filed with the Commission on May 31, 2012 as Exhibit 3.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.1	Indenture dated June 23, 2003, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on August 12, 2003 as Exhibit 4.1 to the Company s Quarterly report on Form 10-Q, which is incorporated herein by reference.
4.2	Indenture dated March 29, 2006, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on March 29, 2006 as Exhibit 4.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.3	First Supplemental Indenture dated March 29, 2006, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on March 29, 2006 as Exhibit 4.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.4	Form of 2.5% Senior Subordinated Convertible Notes due 2013, previously filed with the Commission on March 29, 2006 as Exhibit 4.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.5	Second Supplemental Indenture, dated April 23, 2007, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on April 23, 2007 as Exhibit 4.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.6	Form of 1.875% Senior Subordinated Convertible Notes due 2017, previously filed with the Commission on April 23, 2007 as Exhibit 4.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.1	Form of Indemnification Agreement for Directors and Officers, previously filed with the Commission on October 19, 2010 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.

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10.2	Amended and Restated Severance Plan and Summary Plan Description as originally adopted on January 27, 2004 and amended and restated on May 12, 2009, previously filed with the Commission on July 31, 2009 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated by reference herein.
10.3	Amendment to 1997 Stock Plan, as amended, as adopted March 20, 2002, previously filed with the Commission on March 21, 2002 as Exhibit 99.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.4	Amendment No. 2 to 1997 Stock Plan, as adopted May 5, 2004, previously filed with the Commission on August 9, 2004 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.5	1998 Director Option Plan and forms of agreements thereunder, previously filed with the Commission on May 4, 1999 as Exhibit 10.3 to the Company s Registration Statement on Form S-1 (Registration No. 333-77701), which is incorporated herein by reference.
10.6	Amendment to 1998 Director Plan as adopted March 26, 2003 previously filed with the Commission on May 15, 2003 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.7	Amendment No. 2 to 1998 Director Option Plan, as adopted June 12, 2003 and July 21, 2003, previously filed with the Commission on August 12, 2003 as Exhibit 10.1 to the Company s Quarterly report on Form 10-Q, which is incorporated herein by reference.
10.8	Amendment No. 3 to 1998 Director Option Plan, as adopted May 5, 2004, previously filed with the Commission on August 9, 2004 as Exhibit 10.2 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.9	Amended and Restated 2006 Employee Stock Purchase Plan, as adopted on June 21, 2006, previously filed with the Commission on August 3, 2006 as Exhibit 10.3 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.10	Amended and Restated BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan as adopted on adopted on May 12, 2010, incorporated by reference to Appendix A of the Company s Definitive Proxy Statement on Schedule 14A, as filed with the Commission on March 26, 2010.
10.11	Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted on December 1, 2005 and as amended and restated on January 1, 2009, previously filed with the Commission on December 23, 2008 as Exhibit 10.8 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.12	Summary of Bonus Plan, previously filed with the Commission on February 27, 2009 as Exhibit 10.33 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.13	Amended and Restated Employment Agreement with Jean-Jacques Bienaimé dated January 1, 2009 previously filed with the Commission on December 23, 2008, as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.14	Amended and Restated Employment Agreement with Stephen Aselage dated January 1, 2009 previously filed with the Commission on December 23, 2008 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.

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10.15	Amended and Restated Employment Agreement with Robert A. Baffi dated January 1, 2009 previously filed with the Commission on December 23, 2008, as Exhibit 10.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.16	Amended and Restated Employment Agreement with Jeffrey H. Cooper dated January 1, 2009 previously filed with the Commission on December 23, 2008 as Exhibit 10.5 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.17	Amended and Restated Employment Agreement with G. Eric Davis dated January 1, 2009, previously filed with the Commission on December 23, 2008 as Exhibit 10.6 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.18	Amended and Restated Employment Agreement with Mark Wood dated January 1, 2009 previously filed with the Commission on December 23, 2008 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.19	Employment Agreement with Henry Fuchs, dated March 18, 2009, previously filed with the Commission on March 23, 2009 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.20	Grant Terms and Conditions Agreement between BioMarin Pharmaceutical Inc. and Harbor-UCLA Research and Education Institute dated April 1, 1997, as amended, previously filed with the Commission on July 21, 1999 as Exhibit 10.17 to the Company s Amendment No. 3 to Registration Statement on Form S-1 (Registration No. 333-77701), which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.21	License Agreement dated July 30, 2004, between BioMarin Pharmaceutical Inc. and Daiichi Suntory Pharma Co., Ltd., as amended by Amendment No. 1 to License Agreement dated November 19, 2004, previously filed with the Commission on March 16, 2005 as Exhibit 10.25 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.22	Development, License and Commercialization Agreement dated May 13, 2005, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the Commission on July 6, 2005 as Exhibit 10.1 to the Company s Current Report on Form 8-K/A, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.23	Operating Agreement with Genzyme Corporation, previously filed with the Commission on July 6, 1999 as Exhibit 10.30 to the Company s Amendment No. 2 to Registration Statement on Form S-1 (Registration No. 333-77701), which is incorporated herein by reference.
10.24	License Agreement between BioMarin Pharmaceutical Inc. and Women s and Children s Hospital dated February 7, 2007, previously filed with the Commission on May 3, 2007 as Exhibit 10.7 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.25	Asset Purchase Agreement dated November 30, 2011, by and between a wholly owned subsidiary of BioMarin Pharmaceutical Inc. and SA Pathology, a unit of the Central Adelaide Local Health Network, previously filed with the Commission on February 22, 2012 as Exhibit 10.25 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.

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10.26	Manufacturing, Marketing and Sales Agreement dated as of January 1, 2008, by and among BioMarin Pharmaceutical Inc., Genzyme Corporation and BioMarin/Genzyme LLC previously filed with the Commission on February 28, 2008 as Exhibit 10.30 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.27	Amended and Restated Collaboration Agreement dated as of January 1, 2008, by and among BioMarin Pharmaceutical Inc., Genzyme Corporation and BioMarin/Genzyme LLC previously filed with the Commission on February 28, 2008 as Exhibit 10.31 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.28	Members Agreement dated as of January 1, 2008 by and among BioMarin Pharmaceutical Inc., Genzyme Corporation, BioMarin Genetics Inc., and BioMarin/Genzyme LLC previously filed with the Commission on February 28, 2008 as Exhibit 10.32 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.29	Stock Purchase Agreement by and among BioMarin Pharmaceutical Inc., and LEAD Therapeutics Inc. and the stockholders of LEAD Therapeutics, Inc. dated February 4, 2010, previously filed with the Commission on May 3, 2010 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.30	Stock Purchase Agreement by and between BioMarin Pharmaceutical Inc., Huxley Pharmaceuticals, Inc., and the stockholders of Huxley Pharmaceuticals, Inc., dated October 20, 2009, previously filed with the Commission on February 26, 2010 as Exhibit 10.37 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.31	First Amendment to Stock Purchase Agreement effective as of March 26, 2010, that amends that certain Stock Purchase Agreement, dated as of October 20, 2009 by and among BioMarin Pharmaceutical Inc. and Huxley Pharmaceuticals, Inc. and the stockholders of Huxley previously filed with the Commission on August 4, 2010 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.32	Securities Purchase Agreement dated August 17, 2010 by and among BioMarin Pharmaceutical Inc., ZyStor Therapeutics Inc., the holders of outstanding capital stock and rights to acquire capital stock of ZyStor Therapeutics Inc. and George G. Arida, as the representative of such holders, previously filed with the Commission on August 23, 2010 as Exhibit 2.1 to the Company s Current Report on Form 8-K, which is incorporated by reference herein. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.33	Asset Purchase Agreement dated June 22, 2011 between BioMarin Manufacturing Ireland Limited and Pfizer Biotechnology Ireland, previously filed with the Commission on August 1, 2011 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.

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10.34	Lease Agreement entered into on January 6, 2012 between BioMarin Pharmaceutical Inc. and SR Corporate Center Phase Two, LLC for 770 Lindaro Street, San Rafael, CA, previously filed with the Commission on February 22, 2012 as Exhibit 10.34 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.35	Lease Agreement entered into on January 6, 2012 between BioMarin Pharmaceutical Inc. and SR Corporate Center Phase Two, LLC for 790 Lindaro Street, San Rafael, CA, previously filed with the Commission on February 22, 2012 as Exhibit 10.35 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.36	Severance Agreement and Release of All Claims with Jeffrey H. Cooper, dated February 21, 2012, previously filed with the Commission on February 22, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.37	Employment Agreement with Daniel Spiegelman dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.38	Amendment No. 1 to Employment Agreement with Stephen Aselage dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.39	Amendment No. 1 to Employment Agreement with Robert A. Baffi dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.4 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.40	Amendment No. 1 to Employment Agreement with G. Eric Davis dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.5 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.41	Amendment No. 1 to Employment Agreement with Henry J. Fuchs dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.6 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.42	Amendment No. 1 to Employment Agreement with Mark Wood dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.43	Amendment No. 2 to Employment Agreement with Robert A. Baffi dated May 24, 2012, previously filed with the Commission on May 24, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.44	Amendment No. 2 to Employment Agreement with Henry J. Fuchs dated May 24, 2012, previously filed with the Commission on May 24, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.45	BioMarin Pharmaceutical Inc 2012 Inducement Plan, adopted May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.46	First Amendment to Stock Purchase Agreement dated February 4, 2010 by and among BioMarin Pharmaceutical Inc and LEAD Therapeutics, Inc. and the Stockholders of LEAD Therapeutics dated April 13, 2012, previously filed with the Commission on August 2, 2012 as Exhibit 10.10 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference. The Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Comission.

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10.47	Form of Stock Option Agreement for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan. (As Amended and Restated 2010), previously filed with the Commission on August 2, 2012 as Exhibit 10.11 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.48	Form of Restricted Stock Unit Agreement for the BioMarin Pharmaceutical 2006 Share Incentive Plan. (As Amended and Restated 2010), previously filed with the Commission on August 2, 2012 as Exhibit 10.12 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.49	Form of Stock Option Agreement for the BioMarin Pharmaceutical Inc. 2012 Inducement Plan, previously filed with the Commission on August 2, 2012 as Exhibit 10.13 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.50	Form of Restricted Stock Unit Agreement for the BioMarin Pharmaceutical Inc. 2012 Inducement Plan, previously filed with the Commission on August 2, 2012 as Exhibit 10.14 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.51	Employment Agreement with Jeffrey R. Ajer dated September 5, 2012, previously filed with the Commission on September 5, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.52	Severance Agreement and Release of All Claims with Stephen Aselage, dated September 4, 2012, previously filed with the Commission on September 5, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.53	Amendment No. 1 to Employment Agreement with Daniel Spiegelman dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.54	Amendment No. 1 to Amended and Restated Employment Agreement with Jean-Jacques Bienaime dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.55	Amendment No. 1 to Employment Agreement with Jeffery R. Ajer dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.56	Amendment No. 3 to Employment Agreement with Robert A. Baffi dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.4 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.57	Amendment No. 3 to Employment Agreement with Henry J. Fuchs dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.5 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.58	Amendment No. 2 to Employment Agreement with G. Eric Davis dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.6 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.59	Amendment No. 2 to Employment Agreement with Mark Wood dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.

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10.60*	Second Amendment to Stock Purchase Agreement effective October 26, 2012 by and among BioMarin Pharmaceutical Inc. and Huxley Pharmaceuticals, Inc. and the former stockholders of Huxley. Portions of this document have been redacted pursuant to a Request for Confidential Treatment filed pursuant to the Freedom of Information Act.
21.1*	Subsidiaries of BioMarin Pharmaceutical Inc.
23.1*	Consent of KPMG LLP, Independent Registered Public Accounting Firm for BioMarin Pharmaceutical Inc.
23.2*	Consent of PricewaterhouseCoopers LLP, Independent Accountants for BioMarin/Genzyme LLC.
24.1*	Power of Attorney (Included in Signature Page)
31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1*	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
99.1*	BioMarin/Genzyme LLC Consolidated Financial Statements as of December 31, 2012 and 2011, and for the three years ended December 31, 2012.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Link Document

* Filed herewith
Management contract or compensatory plan or arrangement

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

BIOMARIN PHARMACEUTICAL INC.

Dated: February 26, 2013

By: /s/ DANIEL SPIEGELMAN
Daniel Spiegelman
Executive Vice President and Chief Financial Officer

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POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Jean-Jacques Bienaimé and Daniel Spiegelman, his or her attorney-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to the Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or his substitute or substitutes, may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

Signature	Title	Date
/s/ JEAN-JACQUES BIENAIMÉ **Jean-Jacques Bienaimé**	Chief Executive Officer (Principal Executive Officer)	February 26, 2013
/s/ DANIEL SPIEGELMAN **Daniel Spiegelman**	Executive Vice President and Chief Financial Officer (Principal Financial Officer)	February 26, 2013
/s/ BRIAN R. MUELLER **Brian R. Mueller**	Vice President, Corporate Controller and Chief Accounting Officer (Principal Accounting Officer)	February 26, 2013
/s/ PIERRE LAPALME **Pierre LaPalme**	Chairman and Director	February 26, 2013
/s/ KENNETH BATE **Kenneth Bate**	Director	February 26, 2013
/s/ MICHAEL G. GREY **Michael G. Grey**	Director	February 26, 2013
/s/ ELAINE HERON **Elaine Heron**	Director	February 26, 2013
/s/ V. BRYAN LAWLIS **V. Bryan Lawlis**	Director	February 26, 2013
/s/ ALAN J. LEWIS **Alan J. Lewis**	Director	February 26, 2013
/s/ RICHARD A. MEIER **Richard A. Meier**	Director	February 26, 2013

/s/ WILLIAM YOUNG	Director	February 26, 2013

William Young

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BIOMARIN PHARMACEUTICAL INC.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders

BioMarin Pharmaceutical Inc.:

We have audited the accompanying consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries (the Company) as of December 31, 2012 and 2011, and the related consolidated statements of operations, comprehensive income (loss), stockholders equity, and cash flows for each of the years in the three-year period ended December 31, 2012. These consolidated financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2012 and 2011, and the results of their operations and their cash flows for each of the years in the three-year period ended December 31, 2012, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), BioMarin Pharmaceutical Inc. s internal control over financial reporting as of December 31, 2012, based on criteria established in *Internal Control Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) , and our report dated February 26, 2013 expressed an unqualified opinion on the effectiveness of the Company s internal control over financial reporting.

/s/ KPMG LLP

San Francisco, California

February 26, 2013

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders

BioMarin Pharmaceutical Inc.:

We have audited BioMarin Pharmaceutical Inc. and subsidiaries (the Company) internal control over financial reporting as of December 31, 2012, based on criteria established in *Internal Control Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). The Company s management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management s Annual Report on Internal Control Over Financial Reporting in Item 9A. Our responsibility is to express an opinion on the Company s internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company s internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company s internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company s assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2012, based on criteria established in *Internal Control Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2012 and 2011, and the related consolidated statements of operations, comprehensive income (loss), stockholders equity, and cash flows for each of the years in the three-year period ended December 31, 2012, and our report dated February 26, 2013 expressed an unqualified opinion on those consolidated financial statements.

/s/ KPMG LLP

San Francisco, California

February 26, 2013

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED BALANCE SHEETS

December 31, 2012 and 2011

(In thousands of U.S. dollars, except per share amounts)

	December 31, 2012	December 31, 2011
ASSETS		
Current assets:		
Cash and cash equivalents	$ 180,527	$ 46,272
Short-term investments	270,211	148,820
Accounts receivable, net (allowance for doubtful accounts: $348 and $513, respectively)	109,066	104,839
Inventory	128,695	130,118
Current deferred tax assets	29,454	21,115
Other current assets	25,509	18,638
Total current assets	743,462	469,802
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	1,080	559
Long-term investments	115,993	94,385
Property, plant and equipment, net	284,473	268,971
Intangible assets, net	162,980	180,277
Goodwill	51,543	51,543
Long-term deferred tax assets	225,501	224,677
Other assets	16,611	15,495
Total assets	$ 1,601,643	$ 1,305,709
LIABILITIES AND STOCKHOLDERS EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 147,068	$ 94,125
Convertible debt	23,365	0
Total current liabilities	170,433	94,125
Noncurrent liabilities:		
Long-term convertible debt	324,859	348,329
Long-term contingent acquisition consideration payable	30,618	33,059
Long-term deferred tax liabilities	33,296	37,155
Other long-term liabilities	26,674	19,993
Total liabilities	585,880	532,661
Stockholders equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at December 31, 2012 and 2011: 125,809,162 and 114,789,732 shares issued and outstanding at December 31, 2012 and 2011, respectively.	126	115
Additional paid-in capital	1,561,890	1,197,082
Company common stock held by Nonqualified Deferred Compensation Plan	(6,603)	(3,935)
Accumulated other comprehensive income (loss)	(202)	4,887
Accumulated deficit	(539,448)	(425,101)

Total stockholders equity	1,015,763	773,048
Total liabilities and stockholders equity	$ 1,601,643	$ 1,305,709

The accompanying notes are an integral part of these Consolidated Financial Statements.

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF OPERATIONS

Years Ended December 31, 2012, 2011 and 2010

(In thousands of U.S. dollars, except per share amounts)

	2012	2011	2010
REVENUES:			
Net product revenues	$ 496,497	$ 437,647	$ 369,701
Collaborative agreement revenues	1,955	468	682
Royalty and license revenues	2,271	3,243	5,884
Total revenues	500,723	441,358	376,267
OPERATING EXPENSES:			
Cost of sales (excludes amortization of certain acquired intangible assets)	91,830	84,023	70,285
Research and development	302,218	214,374	147,309
Selling, general and administrative	198,173	175,423	151,723
Intangible asset amortization and contingent consideration	18,717	1,428	6,406
Total operating expenses	610,938	475,248	375,723
INCOME (LOSS) FROM OPERATIONS	(110,215)	(33,890)	544
Equity in the loss of BioMarin/Genzyme LLC	(1,221)	(2,426)	(2,991)
Interest income	2,584	2,934	4,112
Interest expense	(7,639)	(8,409)	(10,818)
Debt conversion expense	0	(1,896)	(13,728)
Net gain from sale of investments	0	0	902
Other income (expense)	(1,787)	60	489
INCOME (LOSS) BEFORE INCOME TAXES	(118,278)	(43,627)	(21,490)
Provision for (benefit from) income taxes	(3,931)	10,209	(227,309)
NET INCOME (LOSS)	$ (114,347)	$ (53,836)	$ 205,819
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.95)	$ (0.48)	$ 2.00
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.95)	$ (0.48)	$ 1.73
Weighted average common shares outstanding, basic	120,271	112,122	103,093
Weighted average common shares outstanding, diluted	120,271	112,122	125,674

The accompanying notes are an integral part of these Consolidated Financial Statements.

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)

Years Ended December 31, 2012, 2011 and 2010

(In thousands of U.S. dollars, except per share amounts)

	2012	2011	2010
NET INCOME (LOSS)	$ (114,347)	$ (53,836)	$ 205,819
OTHER COMPREHENSIVE INCOME (LOSS):			
Net foreign currency gain (loss)	(301)	6	(2)
Available-for-sale securities:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $(140), $(229) and $390 for the years ended December 31, 2012, 2011 and 2010, respectively.	388	(508)	(1,983)
Reclassifications to net income (loss), net of tax impact of $40, $12 and $(148) for the years ended December 31, 2012, 2011 and 2010, respectively.	(110)	27	755
Net Change	278	(481)	(1,228)
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $5,114, $(4,500) and $418 for the years ended December 31, 2012, 2011 and 2010, respectively.	(8,749)	8,163	(3,726)
Reclassifications to net income (loss), net of tax impact of $(2,153), $1,648 and $(473) for the years ended December 31, 2012, 2011 and 2010, respectively.	3,683	(2,989)	4,211
Net Change	(5,066)	5,174	485
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	(5,089)	4,699	(745)
COMPREHENSIVE INCOME (LOSS)	$ (119,436)	$ (49,137)	$ 205,074

The accompanying notes are an integral part of these Consolidated Financial Statements.

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS EQUITY

Years Ended December 31, 2012, 2011 and 2010

(In thousands of U.S. dollars and in thousands of share amounts)

	Common stock			Company Common Stock Held by Nonqualified Deferred			
	Shares	Amount	Additional Paid-in Capital	Compensation Plan	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders Equity
Balance at December 31, 2009	100,962	$ 101	$ 899,950	$ (1,715)	$ 933	$ (577,084)	$ 322,185
Net income						205,819	205,819
Other comprehensive loss					(745)		(745)
Issuance of common stock under Employee Stock Purchase Plan (ESPP)	317		3,777				3,777
Exercise of common stock options	2,040	2	29,461				29,463
Excess tax benefit from stock option exercises			541				541
Conversion of convertible notes	7,213	8	118,234				118,242
Restricted stock vested during the period, net	102		(137)				(137)
Common stock held by Nonqualified Deferred Compensation Plan				(250)			(250)
Stock-based compensation			38,362				38,362
Balance at December 31, 2010	110,634	$ 111	$ 1,090,188	$ (1,965)	$ 188	$ (371,265)	$ 717,257
Net loss						(53,836)	(53,836)
Other comprehensive income					4,699		4,699
Issuance of common stock under ESPP	333		4,411				4,411
Exercise of common stock options	1,925	2	29,710				29,712
Excess tax benefit from stock option exercises			415				415
Conversion of convertible notes	1,761	2	28,980				28,982
Restricted stock vested during the period, net	137		(531)				(531)
Common stock held by Nonqualified Deferred Compensation Plan				(1,970)			(1,970)
Stock-based compensation			43,909				43,909
Balance at December 31, 2011	114,790	$ 115	$ 1,197,082	$ (3,935)	$ 4,887	$ (425,101)	$ 773,048
Net loss						(114,347)	(114,347)
Other comprehensive loss					(5,089)		(5,089)
Issuance of common stock, net of offering costs	6,500	7	235,492				235,499
Issuance of common stock under ESPP	254		5,495				5,495
Exercise of common stock options	4,097	4	77,562				77,566
Excess tax benefit from stock option exercises			473				473
Conversion of convertible notes	6		105				105
Restricted stock vested during the period, net	162		(1,659)				(1,659)
Common stock held by Nonqualified Deferred Compensation Plan				(2,668)			(2,668)
Stock-based compensation			47,340				47,340

Balance at December 31, 2012	125,809	$ 126	$ 1,561,890	$ (6,603)	$ (202)	$ (539,448)	$ 1,015,763

The accompanying notes are an integral part of these Consolidated Financial Statements.

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

Years Ended December 31, 2012, 2011 and 2010

(In thousands of U.S. dollars)

	2012	2011	2010
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net income (loss)	$ (114,347)	$ (53,836)	$ 205,819
Adjustments to reconcile net income (loss) to net cash provided by operating activities:			
Depreciation and amortization	45,295	36,094	27,737
Accretion of discount on investments	4,469	4,036	4,453
Equity in the loss of BioMarin/Genzyme LLC	1,221	2,426	2,991
Stock-based compensation	47,340	43,909	38,362
Impairment of intangible assets	6,707	0	0
Loss on conversion of convertible promissory note	2,000	0	0
Net gain from sale of investments	0	0	(902)
Deferred income taxes	(9,921)	4,363	(230,577)
Excess tax benefit from stock option exercises	(473)	(415)	(541)
Unrealized foreign exchange (loss) gain on forward contracts	(6,529)	7,174	(4,220)
Changes in the fair value of contingent acquisition consideration payable	8,788	(1,795)	3,989
Debt conversion expense	0	1,896	13,728
Changes in operating assets and liabilities:			
Accounts receivable, net	(4,227)	(18,456)	(13,036)
Inventory	1,423	(20,420)	(31,036)
Other current assets	(3,506)	2,543	3,239
Other assets	(4,076)	(837)	(5,326)
Accounts payable and accrued liabilities	37,411	10,109	2,166
Other long-term liabilities	6,034	1,962	1,900
Net cash provided by operating activities	17,609	18,753	18,746
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property, plant and equipment	(44,571)	(73,219)	(49,461)
Maturities and sales of investments	237,837	281,991	206,361
Purchase of available-for-sale investments	(382,168)	(215,429)	(221,659)
Purchase of intellectual property	0	(81,000)	0
Business acquisitions, net of cash acquired	0	0	(32,950)
Investments in BioMarin/Genzyme LLC	(1,743)	(1,903)	(3,633)
Investment in convertible promissory note	(5,000)	0	0
Net cash used in investing activities	(195,645)	(89,560)	(101,342)
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from exercises of stock options and ESPP	81,402	33,592	33,103
Proceeds from public offering of common stock, net	235,499	0	0
Excess tax benefit from stock option exercises	473	415	541
Payment on debt conversion	0	(2,234)	(14,084)
Payment of contingent acquisition consideration payable	(4,405)	(1,894)	(15,861)
Repayment of capital lease obligations	(678)	(879)	(195)
Net cash provided by financing activities	312,291	29,000	3,504
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	134,255	(41,807)	(79,092)

Cash and cash equivalents:

Beginning of period	$ 46,272	$ 88,079	$ 167,171
End of period	$ 180,527	$ 46,272	$ 88,079
SUPPLEMENTAL CASH FLOW DISCLOSURES:			
Cash paid for interest, net of interest capitalized into fixed assets	$ 6,665	$ 7,215	$ 10,077
Cash paid for income taxes	6,582	4,395	3,581
Stock-based compensation capitalized into inventory	4,347	5,298	5,139
Depreciation capitalized into inventory	7,335	6,576	5,088
SUPPLEMENTAL CASH FLOW DISCLOSURES FROM INVESTING AND FINANCING ACTIVITIES:			
Increase (decrease) in accrued liabilities related to fixed assets	$ (511)	$ (320)	$ (4,957)
Conversion of convertible debt	105	29,192	119,562
Deferred offering costs reclassified into additional paid-in capital as a result of conversion of convertible debt	0	210	1,320
Common stock transferred into the Nonqualified Deferred Compensation Plan	0	1,970	250
Equipment acquired through capital leases	0	286	1,313
Increase in asset retirement obligation	886	2,991	0

The accompanying notes are an integral part of these Consolidated Financial Statements.

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

BioMarin Pharmaceutical Inc. (the Company or BioMarin), a Delaware corporation, develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. BioMarin selects product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company s product portfolio is comprised of four approved products and multiple investigational product candidates. The Company s approved products are Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Firdapse (amifampridine phosphate) and Aldurazyme (laronidase).

Through December 31, 2012, the Company had accumulated losses of approximately $539.4 million. Management believes that the Company s cash, cash equivalents and short-term and long-term investments at December 31, 2012 will be sufficient to meet the Company s obligations for at least the next twelve months based on management s current business plans. If the Company elects to increase its spending on development programs significantly above current long-term plans or enters into potential licenses and other acquisitions of complementary technologies, products or companies, the Company may need additional capital. The Company expects to continue to finance future cash needs that exceed its operating activities primarily through its current cash, cash equivalents, short-term and long-term investments, and to the extent necessary, through proceeds from equity or debt financings, loans and collaborative agreements with corporate partners.

The Company is subject to a number of risks, including: the financial performance of Naglazyme, Kuvan, Firdapse and Aldurazyme; the potential need for additional financings; its ability to successfully commercialize its product candidates, if approved; the uncertainty of the Company s research and development efforts resulting in future successful commercial products; obtaining regulatory approval for new products; significant competition from larger organizations; reliance on the proprietary technology of others; dependence on key personnel; uncertain patent protection; dependence on corporate partners and collaborators; and possible restrictions on reimbursement from governmental agencies and healthcare organizations, as well as other changes in the health care industry.

(2) BASIS OF PRESENTATION

Basis of Presentation

These Consolidated Financial Statements have been prepared in accordance with accounting principles generally accepted in the United States (U.S. GAAP) and include the accounts of BioMarin and its wholly owned subsidiaries. All significant intercompany transactions have been eliminated. Management performed an evaluation of the Company s activities through the date of filing of this Annual Report on Form 10-K, and has concluded that there are no subsequent events except for the transaction disclosed in Note 24.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make judgments, estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the dates of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

(3) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Cash and Cash Equivalents

The Company treats liquid investments with original maturities of three months or less when purchased as cash and cash equivalents.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Investments

The Company determines the appropriate classification of its investments in debt and equity securities at the time of purchase and reevaluates such designations at each balance sheet date. All of the Company s securities are classified as available-for-sale and reported in short-term investments or long-term investments. Available-for-sale investments are recorded at fair market value, with unrealized gains or losses included in Accumulated Other Comprehensive Income (Loss) on the Company s Consolidated Balance Sheets, exclusive of other-than-temporary impairment losses, if any. Short-term and long-term investments are comprised of corporate securities, commercial paper, U.S. federal government agency securities and certificates of deposit.

Inventory

Inventories consist of currently marketed products and may contain certain products awaiting regulatory approval. In evaluating the recoverability of inventories produced in preparation for product launches, the Company considers the probability that revenue will be obtained from the future sale of the related inventory together with the status of the product within the regulatory approval process.

The Company values inventory at the lower of cost or net realizable value. The Company determines the cost of inventory using the average-cost method. The Company analyzes its inventory levels quarterly and writes down inventory that has become obsolete, or has a cost basis in excess of its expected net realizable value and inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as Cost of Sales in the Consolidated Statements of Operations.

Investment in BioMarin/Genzyme LLC and Equity in the Loss of BioMarin/Genzyme LLC

The Company accounts for its investment in the joint venture between the Company and Genzyme Corporation (BioMarin/Genzyme LLC) using the equity method. Accordingly, the Company records an increase in its investment for contributions to the joint venture and a reduction in its investment for its 50% share of any losses of the joint venture or disbursements of profits from the joint venture. Equity in the loss of BioMarin/Genzyme LLC includes the Company s 50% share of the joint venture s loss for the period. The investment in BioMarin/Genzyme LLC includes the Company s share of the net equity of the joint venture.

In accordance with Financial Accounting Standards Board (FASB) Accounting Standards Update (ASU) No. 2009-17, *Improvements to Financial Reporting by Enterprises Involved with Variable Interest Entities* (ASU 2009-17) the Company is required to reassess its previous assertion that BioMarin was not the primary beneficiary of BioMarin/Genzyme LLC. Under the guidance, the entity with the power to direct the activities that most significantly impact a variable interest entity s economic performance is the primary beneficiary. The Company has concluded that BioMarin/Genzyme LLC is a variable interest entity, but does not have a primary beneficiary because the power to direct the activities of BioMarin/Genzyme LLC that most significantly impact its performance is shared equally between Genzyme Corporation (Genzyme) and BioMarin through Genzyme s commercialization rights and BioMarin s manufacturing rights.

Property, Plant and Equipment

Property, plant and equipment are stated at cost net of accumulated depreciation. Depreciation is computed using the straight-line method over the related estimated useful lives as presented in the table below. Significant additions and improvements are capitalized, while repairs and maintenance are charged to expense as incurred. Property and equipment purchased for specific research and development projects with no alternative uses are expensed as incurred.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Leasehold improvements	Shorter of life of asset or lease term
Building and improvements	20 years
Manufacturing and laboratory equipment	5 to 15 years
Computer hardware and software	3 to 8 years
Office furniture and equipment	5 years
Vehicles	5 years
Land	Not applicable
Construction-in-progress	Not applicable

Certain of the Company s operating lease agreements include scheduled rent escalations over the lease term, as well as tenant improvement allowances. Scheduled increases in rent expense are recognized on a straight-line basis over the lease term. The difference between rent expense and rent paid is recorded as deferred rent and included in other liabilities in the accompanying Consolidated Balance Sheets. The tenant improvement allowances and free rent periods are recognized as a reduction of rent expense over the lease term on a straight-line basis.

Impairment of Long-Lived Assets

The Company records goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible assets acquired. Goodwill and intangible assets with indefinite lives are not amortized but subject to an annual impairment analysis. Intangible assets with definite lives are amortized over their estimated useful lives on a straight-line basis.

The Company performs its annual impairment review of goodwill and indefinite lived intangibles during the fourth quarter and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. The Company early adopted the provisions of ASU No. 2012-02, *Intangibles Goodwill and Other (Topic 350): Testing Indefinite-Lived Intangible Assets for Impairment* (ASU 2012-02). The early adoption of ASU 2012-02 did not have an impact on Company s financial position or results of operations.

The Company currently operates in one business segment, the biopharmaceutical development and commercialization segment. When reviewing goodwill for impairment, the Company assesses whether goodwill should be allocated to operating levels lower than its single operating segment for which discrete financial information is available and reviewed for decision making purposes. These lower levels are referred to as reporting units. As of December 31, 2012, the Company has only one reporting unit.

The recoverability of the carrying value of the Company s buildings, leasehold improvements for its facilities and equipment depends on the successful execution of the Company s business initiatives and its ability to earn sufficient returns on approved products and product candidates. The Company continually monitors events and changes in circumstances that could indicate carrying amounts of its fixed assets may not be recoverable. When such events or changes in circumstances occur, the Company assesses recoverability by determining whether the carrying value of such assets will be recovered through the undiscounted expected future cash flows. If the future undiscounted cash flows are less than the carrying amount of these assets, the Company recognizes an impairment loss based on the excess of the carrying amount over the fair value of the assets.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Revenue Recognition

The Company recognizes revenue in accordance with FASB Accounting Standards Codification (ASC) Subtopics 605-15, *Revenue Recognition Products* and ASC 605-25, *Revenue Recognition Multiple-Element Arrangements.* The Company s revenues consist of net product revenues from its commercial products, revenues from its collaborative agreement with Merck Serono S.A. (Merck Serono) and other license and royalty revenues. Milestone payments are recognized in full when the related milestone performance goal is achieved and the Company has no future performance obligations related to that payment.

Net Product Revenues The Company recognizes net product revenue when persuasive evidence of an arrangement exists, the product has been delivered to the customer, title and risk of loss have passed to the customer, the price to the buyer is fixed or determinable and collection from the customer is reasonably assured. Product sales transactions are evidenced by customer purchase orders, customer contracts, invoices and/or the related shipping documents. Amounts collected from customers and remitted to governmental authorities, which are primarily comprised of value-added taxes related to Naglazyme and Firdapse sales in foreign jurisdictions, are presented on a net basis in the Company s Consolidated Statements of Operations, in that taxes billed to customers are not included as a component of net product revenues.

The Company receives a 39.5% to 50% royalty on worldwide net Aldurazyme sales by Genzyme depending on sales volume, which is included in net product revenues in the Consolidated Statements of Operations. The Company recognizes a portion of this amount as product transfer revenue when product is released to Genzyme because all of the Company s performance obligations are fulfilled at that point and title to, and risk of loss for, the product has transferred to Genzyme. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay the Company if the product is unsold by Genzyme. The amount of product transfer revenue will eventually be deducted from the calculated royalty earned when the product is sold by Genzyme. The Company records the Aldurazyme royalty revenue based on net sales information provided by Genzyme and records product transfer revenue based on the fulfillment of Genzyme purchase orders in accordance with the terms of the related agreements with Genzyme and when the title and risk of loss for the product is transferred to Genzyme.

The Company sells Naglazyme worldwide, Kuvan in the U.S. and Canada and Firdapse in the EU. In the U.S., Naglazyme and Kuvan are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. The Company also sells Kuvan to Merck Serono at a price near its manufacturing cost, and Merck Serono resells the product to end users outside the U.S., Canada and Japan. The royalty earned from Kuvan product sold by Merck Serono in the EU is included as a component of net product revenues in the period earned and approximates four percent of Merck Serono s world-wide sales. Outside the U.S., Naglazyme and Firdapse are sold to the Company s authorized distributors or directly to government purchasers or hospitals, which act as the end-users. The Company records reserves for rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are recorded. The Company s reserve calculations require estimates, including estimates of customer mix, to determine which sales will be subject to rebates and the amount of such rebates. The Company updates its estimates and assumptions each quarter and records any necessary adjustments to its reserves. The Company records fees paid to distributors as a reduction of revenue.

The Company records allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether an allowance for product returns is required, including market exclusivity of the products based on their orphan drug status, the patient population, the customers limited return rights and the Company s experience with returns. Because of the pricing of the Company s commercial products, the limited number of patients and the customers limited return rights, most Naglazyme, Kuvan and Firdapse customers and retailers carry a limited inventory.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

However, certain international customers, usually government entities, tend to purchase larger quantities of product less frequently. Although such buying patterns may result in revenue fluctuations from quarter to quarter, the Company has not experienced any increased product returns or risk of product returns. The Company relies on historical return rates to estimate returns. Genzyme s contractual return rights for Aldurazyme are limited to defective product. Based on these factors, management has concluded that product returns will be minimal, and the Company has not experienced significant product returns to date. In the future, if any of these factors and/or the history of product returns change, an allowance for product returns may be required.

The Company maintains a policy to record allowances for doubtful accounts for estimated losses resulting from the inability of its customers to make required payments.

Collaborative Agreement Revenues Collaborative agreement revenues include both license revenue and contract research revenue. Nonrefundable up-front license fees where the Company has continuing involvement through research and development collaboration are initially deferred and recognized as collaborative agreement license revenue over the estimated period for which the Company continues to have a performance obligation. Nonrefundable amounts received for shared development costs are recognized as revenue in the period in which the related expenses are incurred.

Royalty and License Revenues Royalty revenues includes royalties on net sales of products with which the Company has no direct involvement and is recognized based on data reported by licensees or sublicensees. Royalties are recognized as earned in accordance with the contract terms at the time the royalty amount is fixed or determinable based on information received from the sublicensees and at the time collectibility is reasonably assured.

Due to the significant role the Company plays in the operations of Aldurazyme and Kuvan, primarily the manufacturing and regulatory activities, as well as the rights and responsibilities to deliver the products to Genzyme and Merck Serono, respectively, the Company elected not to classify these royalties earned as other royalty revenues but instead to include them as a component of Net Product Revenues on the Company s Consolidated Statements of Operations.

Research and Development

Research and development expenses include expenses associated with contract research and development provided by third parties, product manufacturing prior to regulatory approval, clinical and regulatory costs, and internal research and development costs. In instances where the Company enters into agreements with third parties for research and development activities, costs are expensed upon the earlier of when non-refundable amounts are due or as services are performed unless there is an alternative future use of the funds in other research and development projects. Amounts due under such arrangements may be either fixed fee or fee for service and may include upfront payments, monthly payments and payments upon the completion of milestones or receipt of deliverables. The Company accrues costs for clinical trial activities based upon the services received and estimates of related expenses incurred that have yet to be invoiced by the vendors that perform the activities.

Net Income (Loss) Per Share

Basic net income (loss) per share is calculated by dividing net income (loss) by the weighted average shares of common stock outstanding during the period. Diluted net income (loss) per share reflects the potential dilution that would occur if securities or other contracts to issue common stock were exercised or converted into common stock; however, potential common equivalent shares are excluded if their effect is anti-dilutive.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Stock-Based Compensation

The Company uses the Black-Scholes option-pricing model to determine the fair value of stock options and the Company s ESPP awards. The determination of the fair value of stock-based payment awards using an option-pricing model is affected by the Company s stock price as well as assumptions regarding a number of complex and subjective variables. Stock-based compensation expense is recognized on a straight-line basis over the requisite service period for each award. Further, stock-based compensation expense recognized in the Consolidated Statements of Operations is based on awards expected to vest and therefore the amount of expense has been reduced for estimated forfeitures, which are based on historical experience. If actual forfeitures differ from estimates at the time of grant they will be revised in subsequent periods.

The Company uses a lattice model with a Monte Carlo simulation to value restricted stock unit awards with performance and market conditions. This valuation methodology utilizes several key assumptions, including closing price of the Company s stock price on grant date, expected volatility of the Company s stock price, risk-free rates of return, expected dividend yield and estimated total shareholder return.

If factors change and different assumptions are employed in determining the fair value of stock-based awards, the stock-based compensation expense recorded in future periods may differ significantly from what was recorded in the current period (see Note 14 for further information).

Nonqualified Deferred Compensation Plan

The Company s Nonqualified Deferred Compensation Plan (the Deferred Compensation Plan) allows eligible employees, including members of the Company s Board of Directors (the Board), management and certain highly-compensated employees as designated by the Deferred Compensation Plan s administrative committee, to make voluntary deferrals of compensation to specified dates, retirement or death. Participants are permitted to defer portions of their salary, annual cash bonus and restricted stock. The Company is not allowed to make additional direct contributions to the Deferred Compensation Plan on behalf of the participants without further action by the Board.

All of the investments held in the Deferred Compensation Plan are classified as trading securities and recorded at fair value with changes in the investments fair values recognized in earnings in the period they occur. Restricted stock issued and held by the Deferred Compensation Plan is accounted for similarly to treasury stock in that the value of the employer stock is determined on the date the restricted stock vests and the shares are issued into the Deferred Compensation Plan. The restricted stock issued into the Deferred Compensation Plan is recorded as stockholders equity and changes in the fair value of the corresponding liability are recognized in earnings as incurred. The corresponding liability for the Deferred Compensation Plan is included in Accounts Payable and Accrued Liabilities and Other Long-Term Liabilities on the Company s Consolidated Balance Sheets.

Income Taxes

The Company calculates and provides for income taxes in each of the tax jurisdictions in which it operates. Deferred tax assets and liabilities, measured using enacted tax rates, are recognized for the future tax consequences of temporary differences between the tax and financial statement basis of assets and liabilities. A valuation allowance reduces the deferred tax assets to the amount that is more likely than not to be realized. The Company establishes liabilities or reduces assets for uncertain tax positions when the Company believes certain tax positions are not more likely than not of being sustained if challenged. Each quarter, the Company evaluates these uncertain tax positions and adjusts the related tax assets and liabilities in light of changing facts and circumstances.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The Company uses financial projections to support its net deferred tax assets, which contain significant assumptions and estimates of future operations. If such assumptions were to differ significantly, it may have a material impact on the Company s ability to realize its deferred tax assets. At the end of each period, the Company will reassess the ability to realize the deferred tax benefits. If it is more likely than not that the Company would not realize the deferred tax benefits, then all or a portion of the valuation allowance may need to be re-established, which will result in a charge to tax expense.

Foreign Currency and Other Hedging Instruments

The Company has transactions denominated in foreign currencies and, as a result, is exposed to changes in foreign currency exchange rates. The Company manages some of these exposures on a consolidated basis, which results in the netting of certain exposures to take advantage of natural offsets and through the use of foreign currency forward contracts. Gains or losses on net foreign currency hedges are intended to offset gains or losses on the underlying net exposures in an effort to reduce the earnings and cash flow volatility resulting from fluctuating foreign currency exchange rates.

The Company accounts for its derivative instruments as either assets or liabilities on the balance sheet and measures them at fair value. Derivatives that are not defined as hedging instruments are adjusted to fair value through earnings. Gains and losses resulting from changes in fair value are accounted for depending on the use of the derivative and whether it is designated and qualifies for hedge accounting (see Note 10 for further information).

Fair Value of Financial Instruments

The Company discloses the fair value of financial instruments for assets and liabilities for which the value is practicable to estimate. The carrying amounts of all cash equivalents, short-term and long-term investments and forward exchange contracts approximate fair value based upon quoted market prices or discounted cash flows. The fair value of trade accounts receivables, accounts payable and other financial instruments approximates carrying value due to their short-term nature, and which would be considered level 2 items in the fair value hierarchy.

Business Combinations

The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development (IPR&D). In connection with the purchase price allocations for acquisitions, the Company estimates the fair value of contingent payments utilizing a probability-based income approach inclusive of an estimated discount rate.

Contingent Acquisition Consideration Payable

The Company determines the fair value of contingent acquisition consideration payable on the acquisition date using a probability-based income approach utilizing an appropriate discount rate. Each reporting period thereafter, the Company revalues these obligations and records increases or decreases in their fair value as adjustments to Intangible Asset Amortization and Contingent Consideration on the Consolidated Statements of Operations. Changes in the fair value of the contingent acquisition consideration payable can result from adjustments to the estimated probability and assumed timing of achieving the underlying milestones as well as changes to the discount rates and periods.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Comprehensive Income (Loss) and Accumulated Other Comprehensive Income

Comprehensive income (loss) includes net income (loss) and certain changes in stockholders equity that are excluded from net income (loss), such as changes in unrealized gains and losses on the Company s available-for-sale securities, unrealized gains (losses) on foreign currency hedges and changes in the Company s cumulative foreign currency translation account.

Reclassifications and Adjustments

Certain items in the prior year s Consolidated Financial Statements have been reclassified to conform to the current presentation.

(4) RECENT ACCOUNTING PRONOUNCEMENTS

In August 2012, the FASB issued ASU 2012-03, *Technical Amendments and Corrections to SEC Sections: Amendments to SEC Paragraphs Pursuant to SEC Staff Accounting Bulletin No. 114 (SAB No. 114), Technical Amendments Pursuant to SEC Release No. 33-9250, and Corrections Related to FASB Accounting Standards Update 2010-22* (ASU 2012-03). The update amends various SEC paragraphs pursuant to the issuance of SAB No. 114 and is effective upon issuance. The adoption of the amended guidance in ASU 2012-03 did not have a significant impact on the Company s Consolidated Financial Statements.

In October 2012, the FASB issued ASU 2012-04, *Technical Corrections and Improvements* (ASU 2012-04). The amendments in this update cover a wide range of Topics in the Accounting Standards Codification including technical corrections and improvements to the Accounting Standards Codification and conforming amendments related to fair value measurements. The amendments in ASU 2012-04 are effective for fiscal periods beginning after December 15, 2012, which for the Company means January 1, 2013. The adoption of ASU 2012-04 is not expected to have a material impact on the Company s Consolidated Financial Statements.

(5) SHORT-TERM AND LONG-TERM INVESTMENTS

All investments were classified as available-for-sale at December 31, 2012 and 2011. The principal amounts of short-term and long-term investments by contractual maturity are summarized in the tables below:

	Contractual Maturity Date for the Years Ending December 31,			Total Book Value at December 31, 2012	Unrealized Gain (Loss)	Aggregate Fair Value at December 31, 2012
	2013	2014	2015			
Certificates of deposit	$ 36,603	$ 12,138	$ 0	$ 48,741	$ 13	$ 48,754
Corporate debt securities	221,954	40,447	54,308	316,709	191	316,900
Corporate equity securities	3,000	0	0	3,000	(67)	2,933
U.S. Government agency securities	8,512	2,500	6,500	17,512	5	17,517
Greek government-issued bonds	0	0	48	48	52	100
Total	$ 270,069	$ 55,085	$ 60,856	$ 386,010	$ 194	$ 386,204

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

	Contractual Maturity Date for the Years Ending December 31,			Total Book Value at	Unrealized Gain	Aggregate Fair Value at
	2012	2013	2014	December 31, 2011	(Loss)	December 31, 2011
Certificates of deposit	$ 38,547	$ 17,195	$ 0	$ 55,742	$ 13	$ 55,755
Commercial paper	24,730	0	0	24,730	(9)	24,721
Corporate debt securities	85,595	40,899	3,100	129,594	53	129,647
U.S. Government agency securities	0	32,877	0	32,877	13	32,890
Greek government-issued bonds	0	192	0	192	0	192
Total	$ 148,872	$ 91,163	$ 3,100	$ 243,135	$ 70	$ 243,205

The Company completed an evaluation of its investments and determined that it did not have any other-than-temporary impairments as of December 31, 2012. The investments are in institutions that have strong financial ratings and management expects full recovery of the carrying amounts.

See Note 12 for additional discussion regarding the fair value of Greek government-issued bonds held by the Company.

The aggregate amounts of unrealized losses and related fair value of investments with unrealized losses as of December 31, 2012 and 2011 were as follows:

	Less Than 12 Months to Maturity		12 Months or More to Maturity		Totals at December 31, 2012	
	Aggregate Fair Value	Unrealized Losses	Aggregate Fair Value	Unrealized Losses	Aggregate Fair Value	Unrealized Losses
Certificates of deposit	$ 7,974	$ (1)	$ 2,974	$ 0	$ 10,948	$ (1)
Corporate debt securities	59,375	(44)	43,559	(167)	102,934	(211)
Corporate equity securities	2,933	(67)	0	0	2,933	(67)
Total	$ 70,282	$ (112)	$ 46,533	$ (167)	$ 116,815	$ (279)

	Less Than 12 Months to Maturity		12 Months or More to Maturity		Totals at December 31, 2011	
	Aggregate Fair Value	Unrealized Losses	Aggregate Fair Value	Unrealized Losses	Aggregate Fair Value	Unrealized Losses
Certificates of deposit	$ 7,489	$ 0	$ 8,118	$ (5)	$ 15,607	$ (5)
Commercial paper	7,474	(12)	0	0	7,474	(12)
Corporate debt securities	26,840	(184)	9,571	(29)	36,411	(213)
U.S. Government agency securities	0	0	11,252	(1)	11,252	(1)
Total	$ 41,803	$ (196)	$ 28,941	$ (35)	$ 70,744	$ (231)

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(6) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	December 31,	
	2012	2011
Intangible assets:		
Finite-lived intangible assets	$ 118,242	$ 118,242
Indefinite-lived intangible assets	63,689	70,396
Gross intangible assets:	181,931	188,638
Less: Accumulated amortization	(18,951)	(8,361)
Net carrying value	$ 162,980	$ 180,277

Finite-Lived Intangible Assets

The following table summarizes the annual amortization of the finite-lived intangible assets through 2023:

	Net Balance at December 31, 2012	Estimated Useful Life	Remaining Life	Annual Amortization
Naglazyme intellectual property	$ 73,688	12 years	10.9 years	$ 6,750
EU marketing rights for Firdapse	23,364	10 years	7.3 years	3,223
License payment for Kuvan FDA Approval	648	7 years	1.9 years	332
License payment for Kuvan EMEA Approval	1,591	10 years	5.9 years	269
Total	$ 99,291			$ 10,574

On November 30, 2011, the Company entered into an asset purchase agreement to purchase certain intellectual property from SA Pathology, a unit of the Central Adelaide Local Health Network located in Adelaide, Australia, for an upfront cash payment of $81.0 million. The intellectual property purchased by the Company includes issued and pending patents related to the purified form of Naglazyme and the method of using the enzyme in the treatment of Mucopolysaccharidosis VI, which expire between 2022 and 2023. Prior to this purchase, the Company licensed this intellectual property from SA Pathology and paid to them a 5% royalty on net sales of Naglazyme. In the years ended December 31, 2012 and 2011, the Company recognized amortization expense of $6.8 million and $0.5 million, respectively, related to the Naglazyme intellectual property as a component of cost of sales in the Consolidated Statements of Operations.

The Firdapse intangible assets consist of Firdapse product technology acquired as part of the Huxley Pharmaceuticals Inc. (Huxley) acquisition in the fourth quarter of 2009, for which the EMEA granted marketing approval in December 2009. The EMEA did not enable the commercial launch of Firdapse until April 2010, at which time the Company began amortizing the European product technology at an annual rate of $3.2 million. As a result of the EMEA approval of Firdapse, the Company made license payments of $2.0 million to a third-party in 2010 increasing the gross value of the European marketing rights for Firdapse by $2.0 million. In each of the years ended December 31, 2012, 2011 and 2010, the Company recognized $3.2 million, $3.2 million and $2.4 million, respectively, of amortization expense related to the EU marketing rights for Firdapse as a component of Intangible Asset Amortization and Contingent Consideration in the Consolidated Statement of Operations.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The Kuvan intangible assets relate to license payments made to third parties as a result of the FDA approval of Kuvan in December 2007 and the EMEA approval in December 2008, which resulted in a $2.7 million addition to the Kuvan intangible assets. At December 31, 2012 and 2011, Kuvan intangible assets totaled a gross value of $5.0 million. In each of the years ended December 31, 2012, 2011 and 2010, the Company recognized $0.6 million of amortization expense related to the Kuvan intangible assets as a component of Cost of Sales in the Consolidated Statements of Operations.

Indefinite-Lived Intangible Assets

Indefinite-lived intangible assets consist of IPR&D assets related to both early and late stage product candidates purchased in the acquisitions of Huxley, LEAD Therapeutics, Inc. (LEAD) and ZyStor Therapeutics, Inc. (ZyStor). In estimating fair value of the IPR&D assets, the Company compensated for the differing phases of development of each asset by probability-adjusting its estimation of the expected future cash flows associated with each asset. The Company then determined the present value of the expected future cash flows. The projected cash flows from the IPR&D assets were based on key assumptions such as estimates of revenues and operating profits related to the feasibility and timing of achievement of development, regulatory and commercial milestones, expected costs to develop the IPR&D into commercially viable products and future expected cash flows from product sales.

Indefinite-lived intangible assets consisted of the following:

	December 31,	
	2012	2011
In-Process Research and Development:		
U.S. marketing rights for Firdapse	$ 0	$ 6,707
BMN-673 acquired through LEAD	36,089	36,089
BMN-701 acquired through ZyStor	25,010	25,010
Other acquired pre-clinical compounds	2,590	2,590
Net carrying value	$ 63,689	$ 70,396

Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate a reduction in the fair value of the IPR&D assets below their respective carrying amounts. During the first quarter of 2012, the Company recorded an impairment charge of $6.7 million related to certain Firdapse IPR&D assets. These IPR&D assets were associated with marketing rights in the U.S. The Company was exploring strategic options for the Firdapse U.S. program, including the potential outlicense of rights in the U.S. In March 2012, the Company recognized an impairment charge based on the status of business development efforts at the time and the related discounted cash flow projections that no longer supported the carrying-value of the IPR&D intangible assets. The impairment charge was included in Intangible Asset Amortization and Contingent Consideration on the Consolidated Statements of Operations for the three years ended December 31, 2012. Additionally, during the fourth quarter of 2012, the Company performed its annual impairment review and determined that no additional impairments existed as of December 31, 2012.

If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(7) PROPERTY, PLANT AND EQUIPMENT

Property, plant and equipment, net consisted of the following:

	December 31,	
	2012	2011
Leasehold improvements	$ 65,918	$ 49,456
Building and improvements	144,700	141,484
Manufacturing and laboratory equipment	79,915	72,039
Computer hardware and software	56,011	48,566
Furniture and equipment	11,143	7,679
Land	11,608	11,608
Construction-in-progress	64,300	53,884
	433,595	384,716
Less: Accumulated depreciation	(149,122)	(115,745)
Total property, plant and equipment, net	$ 284,473	$ 268,971

Depreciation expense for the years ended December 31, 2012, 2011 and 2010 was $34.9 million, $31.9 million and $23.3 million, respectively, of which $7.3 million, $6.6 million and $5.1 million was capitalized into inventory, respectively.

Capitalized interest related to the Company s property, plant and equipment purchases for the years ended December 31, 2012 and 2011 was insignificant compared to the year ended December 31, 2010 when capitalized interest was $0.7 million.

(8) INVENTORY

Inventory consisted of the following:

	December 31,	
	2012	2011
Raw materials	$ 11,943	$ 12,145
Work-in-process	71,443	75,903
Finished goods	45,309	42,070
Total inventory	$ 128,695	$ 130,118

Inventory as of December 31, 2012 includes $12.0 million of product manufactured using certain process and specification changes that have not yet received regulatory approval. The process and specification changes are required to be approved by the FDA before the product can be sold commercially, however the Company expects to receive FDA approval and realize the costs of the inventory through future sales.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(9) SUPPLEMENTAL BALANCE SHEET INFORMATION

Accounts payable and accrued liabilities consisted of the following:

	December 31,	
	2012	2011
Accounts payable	$ 23,993	$ 12,239
Accrued accounts payable	43,156	23,849
Accrued vacation expense	8,403	6,530
Accrued compensation expense	27,530	17,619
Accrued interest expense	1,306	1,300
Accrued royalties payable	4,991	5,866
Accrued rebates payable	9,625	6,025
Other accrued operating expenses	6,179	9,259
Current portion of nonqualified deferred compensation liability	6,440	682
Value added taxes payable	2,072	3,165
Current portion of contingent acquisition consideration payable	10,764	5,555
Other	2,609	2,036
Total accounts payable and accrued liabilities	$ 147,068	$ 94,125

The roll forward of significant estimated accrued rebates, reserve for cash discounts and allowance for doubtful accounts for 2012, 2011 and 2010 was as follows:

	Balance at Beginning of Period	Provision for Current Period Sales	Provision/ (Reversals) for Prior Period Sales	Actual Charges Related to Current Period Sales	Actual Charges Related to Prior Period Sales	Balance at End of Period
Year ended December 31, 2012:						
Accrued rebates	$ 6,025	$ 16,449	$ (434)	$ (8,193)	$ (4,222)	$ 9,625
Reserve for cash discounts	342	4,214	0	(4,184)	0	372
Allowance for doubtful accounts	513	0	(165)	0	0	348
Year ended December 31, 2011:						
Accrued rebates	$ 5,899	$ 14,369	$ (639)	$ (10,042)	$ (3,562)	$ 6,025
Reserve for cash discounts	304	3,543	0	(3,209)	(296)	342
Allowance for doubtful accounts	64	0	1,053	0	(604)	513
Year ended December 31, 2010:						
Accrued rebates	$ 4,786	$ 11,835	$ (1,859)	$ (6,537)	$ (2,326)	$ 5,899
Reserve for cash discounts	259	2,987	0	(2,723)	(219)	304

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(10) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

Foreign Currency Exchange Rate Exposure

The Company uses forward foreign currency exchange contracts to hedge certain operational exposures resulting from changes in foreign currency exchange rates. Such exposures result from portions of the Company s forecasted revenues and operating expenses being denominated in currencies other than the U.S. dollar, primarily the Euro and Brazilian Real, respectively.

The Company designates certain of these forward foreign currency exchange contracts as hedging instruments and enters into some forward foreign currency exchange contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward foreign currency exchange contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from Naglazyme product revenues, Aldurazyme royalty revenues, operating expenses and net asset or liability positions designated in currencies other than the U.S. dollar. The fair values of forward foreign currency exchange contracts are estimated using current exchange rates and interest rates, and take into consideration the current creditworthiness of the counterparties or the Company, as applicable. Details of the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations follow below. See Note 12 for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At December 31, 2012, the Company had 88 forward foreign currency exchange contracts outstanding to sell a total of 50.6 million Euros and five forward foreign currency exchange contracts outstanding to buy 6.0 million Brazilian Reais with expiration dates ranging from January 2013 through May 2014. These hedges were entered into in order to protect against the fluctuations in revenue associated with Euro denominated Naglazyme, Firdapse and Aldurazyme sales and operating expenses denominated in the Brazilian Real. The Company has formally designated these forward foreign currency exchange contracts as cash flow hedges and expects them to be highly effective within the meaning of FASB ASC Subtopic 815-30, *Derivatives and Hedging-Cash Flow Hedges,* in offsetting fluctuations in revenues denominated in Euros and operating expenses denominated in the Brazilian Real related to changes in the foreign currency exchange rates.

The Company also enters into forward foreign currency exchange contracts that are not designated as hedges for accounting purposes. The changes in fair value of these forward foreign currency exchange contracts are included as a part of Selling, General and Administrative expense in the Consolidated Statements of Operations. At December 31, 2012, separate from the 93 contracts discussed above, the Company had one outstanding forward foreign currency exchange contract to sell 32.4 million Euros that was not designated as a hedge for accounting purposes that matured on January 31, 2013.

The maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency cash flows through forward foreign currency exchange contracts is through May 2014. Over the next twelve months, the Company expects to reclassify $0.4 million from accumulated other comprehensive income to earnings as the forecasted revenue transactions and operating expenses occur.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The fair value carrying amounts of the Company s derivative instruments were as follows:

	Asset Derivatives December 31, 2012		Liability Derivatives December 31, 2012	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 1,463	Accounts payable and accrued liabilities	$ 1,078
Forward foreign currency exchange contracts	Other assets	0	Other long-term liabilities	368
Total		$ 1,463		$ 1,446
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 84	Accounts payable and accrued liabilities	$ 0
Total		84		0
Total value of derivative contracts		$ 1,547		$ 1,446

	Asset Derivatives December 31, 2011		Liability Derivatives December 31, 2011	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 4,705	Accounts payable and accrued liabilities	$ 189
Forward foreign currency exchange contracts	Other assets	1,977	Other long-term liabilities	26
Total		$ 6,682		$ 215
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 0	Accounts payable and accrued liabilities	$ 5
Total		0		5
Total value of derivative contracts		$ 6,682		$ 220

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The effect of the Company s derivative instruments on the Consolidated Financial Statements for the years ended December 31, 2012, 2011 and 2010 was as follows:

	Forward Foreign Currency Exchange Contracts		
	2012	2011	2010
Derivatives Designated as Hedging Instruments:			
Net gain (loss) recognized in Other Comprehensive Income (OCI) (1)	$ (8,027)	$ 8,026	$ 540
Net gain (loss) reclassified from accumulated OCI into income (2)	5,836	(4,637)	4,684
Net gain (loss) recognized in income (3)	927	(1,486)	285
Derivatives Not Designated as Hedging Instruments:			
Net gain (loss) recognized in income (4)	$ 674	$ 674	$ 1,512

(1) Net change in the fair value of the effective portion classified as OCI
(2) Effective portion classified as net product revenue
(3) Ineffective portion and amount excluded from effectiveness testing classified as selling, general and administrative expense
(4) Classified as selling, general and administrative expense

At December 31, 2012, 2011 and 2010, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a loss of $0.2 million, a gain of $8.0 million and a loss of $0.2 million, respectively.

The Company is exposed to counterparty credit risk on all of its derivative financial instruments. The Company has established and maintained strict counterparty credit guidelines and enters into hedges only with financial institutions that are investment grade or better to minimize the Company s exposure to potential defaults. The Company does not require collateral to be pledged under these agreements.

(11) CONVERTIBLE DEBT

In April 2007, the Company sold approximately $324.9 million of senior subordinated convertible notes due 2017 (the 2017 Notes). The debt was issued at face value and bears interest at the rate of 1.875% per annum, payable semi-annually in cash. The debt is convertible, at the option of the holder, at any time prior to maturity or redemption, into shares of the Company s common stock at a conversion price of approximately $20.36 per share, subject to adjustment in certain circumstances. The debt does not include a call provision and the Company is unable to unilaterally redeem the debt prior to maturity on April 23, 2017. The Company also must repay the debt if there is a qualifying change in control or termination of trading of its common stock. If a change of control occurs, the Company will pay a make whole premium by increasing the conversion rate applicable to the notes.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

In connection with the placement of the 2017 Notes, the Company paid approximately $8.5 million in offering costs, which have been deferred and are included in other assets. The deferred offering costs are being amortized as interest expense over the life of the debt, and in each of the years ended December 31, 2012, 2011 and 2010 the Company recognized amortization expense of $0.9 million.

In March 2006, the Company sold $172.5 million of senior subordinated convertible notes due 2013 (the 2013 Notes), of which $23.4 million remains outstanding at December 31, 2012. The debt was issued at face value and bears interest at the rate of 2.5% per annum, payable semi-annually in cash. The debt is convertible, at the option of the holder, at any time prior to maturity or redemption, into shares of the Company s common stock at a conversion price of approximately $16.58 per share, subject to adjustment in certain circumstances. The debt does not include a call provision and the Company is unable to unilaterally redeem the debt prior to maturity on March 29, 2013. The Company also must repay the debt if there is a qualifying change in control or termination of trading of its common stock. If a change of control occurs, the Company will pay a make whole premium by increasing the conversion rate applicable to the notes.

In connection with the placement of the 2013 Notes, the Company paid approximately $5.5 million in offering costs, which have been deferred and are included in other assets. The deferred offering costs are being amortized as interest expense over the life of the debt. The Company recognized amortization expense of approximately $0.1 million for the year ended December 31, 2012, compared to $0.2 million and $0.7 million for the years ended December 31, 2011 and 2010, respectively. The decrease in amortization expense for the year ended December 31, 2012 was attributed to the conversion of $29.2 million and $119.6 million in aggregate principal of the 2013 Notes in September 2011 and November 2010, respectively.

In September 2011, the Company entered into separate agreements with nine of the existing holders of its 2013 Notes pursuant to which such holders converted $29.2 million in aggregate principal amount of the 2013 Notes into 1,760,178 shares of the Company s common stock. In addition to issuing the requisite number of shares of the Company s common stock pursuant to the 2013 Notes, the Company paid the holders future interest of approximately $1.1 million along with an aggregate of approximately $0.8 million related to varying cash premiums for agreeing to convert the 2013 Notes, which was recognized in total as Debt Conversion Expense on the Consolidated Statement of Operations for the year ended December 31, 2011. Additionally, the Company reclassified $0.2 million of deferred offering costs to additional paid-in capital in connection with the conversion of the 2013 Notes. During 2012 and 2011, certain note holders voluntarily exchanged an insignificant number of convertible notes for shares of the Company s common stock.

In November 2010, the Company entered into separate agreements with nine of the existing holders of its 2013 Notes pursuant to which such holders converted $119.6 million in aggregate principal amount of the 2013 Notes into 7,213,379 shares of the Company s common stock. In addition to issuing the requisite number of shares of the Company s common stock pursuant to the 2013 Notes, the Company paid the holders future interest of approximately $7.2 million along with an aggregate of approximately $6.5 million related to varying cash premiums for agreeing to convert the 2013 Notes, which was recognized in total as Debt Conversion Expense on the Company s Consolidated Statement of Operations for the year ended December 31, 2010. Additionally, the Company reclassified $1.3 million of deferred offering costs to additional paid-in capital in connection with the conversion of the 2013 Notes.

Interest expense on the Company s convertible debt for the year ended December 31, 2012 was $6.7 million, compared to $7.4 million and $10.0 million for the years ended December 31, 2011 and 2010, respectively. The decrease in interest expense related to the Company s convertible debt in 2012, compared to 2011 and 2010 was attributed to the conversion of $29.2 million and $119.6 million in aggregate principal of the 2013 Notes in September 2011 and November 2010, respectively.

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(12) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including available-for-sale fixed income securities and foreign currency derivatives. The tables below present the fair value of these financial assets and liabilities determined using the following input levels.

	Fair Value Measurements at December 31, 2012			
	Quoted Price in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Overnight deposits	$ 54,018	$ 0	$ 0	$ 54,018
Money market instruments	0	126,509	0	126,509
Total cash and cash equivalents	$ 54,018	$ 126,509	$ 0	$ 180,527
Available-for-sale securities:				
Short-term:				
Certificates of deposit	$ 0	$ 36,615	$ 0	$ 36,615
Corporate debt securities	0	222,147	0	222,147
Corporate equity securities	0	2,933	0	2,933
U.S. Government agency securities	0	8,516	0	8,516
Long-term:				
Certificates of deposit	0	12,139	0	12,139
Corporate debt securities	0	94,753	0	94,753
U.S. Government agency securities	0	9,001	0	9,001
Greek government-issued bonds	0	100	0	100
Total available-for-sale securities	$ 0	$ 386,204	$ 0	$ 386,204
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 2,052	$ 0	$ 2,052
Forward foreign currency exchange contract asset (1)	0	1,547	0	1,547
Restricted investments (2)	0	2,243		2,243
Total other current assets	$ 0	$ 5,842	$ 0	$ 5,842
Other Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 2,375	$ 0	$ 2,375
Restricted investments (2)	0	3,492	0	3,492
Total other assets	$ 0	$ 5,867	$ 0	$ 5,867
Total assets	$ 54,018	$ 524,422	$ 0	$ 578,440

Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 6,440	$ 0	$ 0	$ 6,440
Forward foreign currency exchange contract liability (1)	0	1,078	0	1,078
Contingent acquisition consideration payable	0	0	10,764	10,764
Asset retirement obligation	0	0	1,685	1,685
Total current liabilities	$ 6,440	$ 1,078	$ 12,449	$ 19,967
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 5,041	$ 4,427	$ 0	$ 9,468
Forward foreign currency exchange contract liability (1)	0	368	0	368
Contingent acquisition consideration payable	0	0	30,618	30,618
Asset retirement obligation	0	0	2,192	2,192
Total other long-term liabilities	$ 5,041	$ 4,795	$ 32,810	$ 42,646
Total liabilities	$ 11,481	$ 5,873	$ 45,259	$ 62,213

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

	Fair Value Measurements at December 31, 2011			
	Quoted Price in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Overnight deposits	$ 44,212	$ 0	$ 0	$ 44,212
Money market instruments	0	2,060	0	2,060
Total cash and cash equivalents	$ 44,212	$ 2,060	$ 0	$ 46,272
Available-for-sale securities:				
Short-term:				
Certificates of deposit	$ 0	$ 38,564	$ 0	$ 38,564
Commercial paper	0	24,721	0	24,721
Corporate debt securities	0	85,535	0	85,535
Long-term:				
Certificates of deposit	0	17,191	0	17,191
Corporate debt securities	0	44,112	0	44,112
U.S. Government agency securities	0	32,890	0	32,890
Greek government-issued bonds	0	192	0	192
Total available-for-sale securities	$ 0	$ 243,205	$ 0	$ 243,205
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 146	$ 0	$ 146
Forward foreign currency exchange contract asset (1)	0	4,705	0	4,705
Total other current assets	$ 0	$ 4,851	$ 0	$ 4,851
Other Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 3,359	$ 0	$ 3,359
Forward foreign currency exchange contract asset (1)	0	1,977	0	1,977
Total other assets	$ 0	$ 5,336	$ 0	$ 5,336
Total assets	$ 44,212	$ 255,452	$ 0	$ 299,664
Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 0	$ 682	$ 0	$ 682
Forward foreign currency exchange contract liability (1)	0	194	0	194
Contingent acquisition consideration payable	0	0	5,555	5,555
Total current liabilities	$ 0	$ 876	$ 5,555	$ 6,431
Other long-term liabilities:				

Nonqualified Deferred Compensation Plan liability	$ 5,945	$ 2,823	$ 0	$ 8,768
Forward foreign currency exchange contract liability (1)	0	26	0	26
Contingent acquisition consideration payable	0	0	33,059	33,059
Asset retirement obligation	0	0	2,991	2,991
Total other long-term liabilities	$ 5,945	$ 2,849	$ 36,050	$ 44,844
Total liabilities	$ 5,945	$ 3,725	$ 41,605	$ 51,275

(1) See Note 10 for further information regarding the derivative instruments.

(2) The restricted investments secure the Company s irrevocable standby letter of credit obtained in connection with the Company s new corporate facility lease agreements and certain commercial agreements.

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(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

There were no transfers between levels during the years ended December 31, 2012 and 2011.

The Company s level 2 securities are valued using third-party pricing sources. The pricing services utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs.

The Company validates the prices provided by its third-party pricing services by understanding the models used, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming those securities traded in active markets. Due to the continued volatility associated with market conditions in Greece and reduced trading activity in its sovereign debt, the Company classified its Greek government-issued bonds as level 2 on December 31 2012 and 2011. See Note 5 for further information regarding the Company s financial instruments.

Liabilities measured at fair value using level 3 inputs were comprised of contingent acquisition consideration payable and asset retirement obligations.

The Company s contingent acquisition consideration payable is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probabilities, the estimated timing of when a milestone may be attained and assumed discount periods and rates. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management s revision of key assumptions, will be recorded in Intangible Asset Amortization and Contingent Consideration on the Consolidated Statements of Operations.

Contingent acquisition consideration payable at December 31, 2011	$ 38,614
Changes in the fair value of the contingent acquisitions	8,788
Payments of contingent acquisition consideration payable to former stockholders of LEAD	(6,020)
Contingent acquisition consideration payable at December 31, 2012	$ 41,382

Under certain of the Company s lease agreements, the Company is contractually obligated to return leased space to its original condition upon termination of the lease agreement. The Company records an asset retirement obligation liability and a corresponding capital asset in an amount equal to the estimated fair value of the obligation when estimatable. In subsequent periods, for each such lease, the Company records interest expense to accrete the asset retirement obligation liability to full value and depreciates each capitalized asset retirement obligation asset, both over the term of the associated lease agreement. The Company s asset retirement obligations were $3.9 million and $3.0 million at December 31, 2012 and 2011, respectively.

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The Company acquired intangible assets as a result of various business acquisitions. The estimated fair value of these long-lived assets was measured using level 3 inputs as of the acquisition date.

(13) STOCKHOLDERS EQUITY

In June 2012, the Company sold 6.5 million shares of its common stock at a price of $36.28 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the Securities and Exchange Commission. The Company received cash proceeds of approximately $235.5 million from this public offering.

2012 Inducement Plan

On May 8, 2012, the Company s Board of Directors approved the 2012 Inducement Plan (2012 Inducement Plan), which provides for grants of up to 750,000 share-based awards to new employees, including grants of restricted stock units (RSUs) and grants of options to purchase common stock at a price equal to the fair market value of such shares on the date of grant. The awards are substantially similar to those granted under the Company s 2006 Share Incentive Plan as amended and restated on March 22, 2010 (2006 Share Incentive Plan). The 2012 Inducement Plan expires in March 2013.

Share Incentive Plan

BioMarin s Share Incentive Plan, which replaced the Company s previous stock option plans (the 1997 Stock Plan and the 1998 Directors Options Plan), provides for grants of options to employees to purchase common stock at the fair market value of such shares on the grant date, as well as other forms of equity compensation. As of December 31, 2012, awards issued under the Share Incentive Plan include both stock options and restricted stock units. Stock option awards granted to employees generally vest over a four-year period on a cliff basis six months after the grant date and then monthly thereafter. The term of the outstanding options is generally ten years. Restricted stock units granted to employees generally vest in a straight-line annually over a four-year period after the grant date. Restricted stock units granted to directors generally vest in full one year after the grant date.

As of December 31, 2012, options to purchase approximately 0.2 million, 12.8 million and 0.9 million shares were outstanding under the 2012 Inducement Plan, the Share Incentive Plan, and the Company s previous plans, respectively.

Employee Stock Purchase Plan

Under BioMarin s ESPP, which was approved in June 2006 and replaced the Company s previous plan, employees meeting specific employment qualifications are eligible to participate and can purchase shares on established dates semi-annually through payroll deductions at the lower of 85% of the fair market value of the stock at the commencement or each purchase date of the offering period. Each offering period will span up to two years. The ESPP permits eligible employees to purchase common stock through payroll deductions for up to 10% of qualified compensation, up to an annual limit of $25,000. The ESPP is intended to qualify as an employee stock purchase plan under Section 423 of the Internal Revenue Code. During 2012, the Company issued 254,285 shares under the Employee Stock Purchase Plan. As of December 31, 2012 there were approximately 0.6 million shares reserved for future issuance.

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Board of Director Grants

An initial option is granted to each new outside member of BioMarin s Board of Directors to purchase 30,000 shares of common stock at the fair value on the date of the grant. Until January 2007, on each anniversary date of becoming a director, each outside member was granted options to purchase 30,000 shares of common stock at the fair market value on such date. Currently, on the date of each annual meeting of stockholders, other than newly elected directors, each outside director is granted options for the purchase of 15,000 shares of common stock and 2,500 restricted stock units. The options vest over one year and have a term of ten years. The restricted stock units vest on the one year anniversary of the date of grant.

Stockholders Rights Plan

On May 30, 2012, the Company entered into Amendment No. 1 (the Amendment) to the Amended and Restated Rights Agreement, dated February 27, 2009, between the Company and Computershare Shareowner Services LLC (formerly known as Mellon Investor Services LLC) as Rights Agent (the Rights Agreement). The Amendment accelerated the final expiration date of the Company s preferred share purchase rights (the Rights) under the Rights Agreement from September 23, 2012 to May 30, 2012. As a result, each outstanding share of the Company s common stock is no longer accompanied by a Right. The holders of common stock were not entitled to any payment as a result of the expiration of the Rights Agreement and the Rights issued thereunder.

(14) STOCK-BASED COMPENSATION

The following table summarizes activity under the Company s stock option plans, including the 2012 Inducement Plan and those suspended upon the adoption of the Share Incentive Plan. All option grants presented in the table had exercise prices not less than the fair value of the underlying common stock on the grant date:

	Year Ended December 31,					
	2012		2011		2010	
	Shares	Weighted-Average Exercise Price	Shares	Weighted-Average Exercise Price	Shares	Weighted-Average Exercise Price
Outstanding, beginning of year	16,319,150	$ 22.33	14,900,241	$ 20.08	14,046,895	$ 19.04
Granted	2,296,570	$ 37.70	3,867,464	$ 27.89	3,554,932	$ 21.63
Exercised	(4,097,279)	$ 18.95	(1,923,455)	$ 15.42	(2,041,980)	$ 14.44
Expired and forfeited	(648,840)	$ 26.14	(525,100)	$ 24.70	(659,606)	$ 23.80
Outstanding, end of year	13,869,601	$ 25.69	16,319,150	$ 22.33	14,900,241	$ 20.08
Options expected to vest	4,444,406	$ 28.83	5,506,909	$ 23.61	5,125,725	$ 20.92
Exercisable, end of year	8,722,417	$ 23.30	9,904,117	$ 21.06	8,880,548	$ 19.15
Weighted-average grant date fair value of options granted during the year		$ 16.98		$ 13.60		$ 11.25

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The aggregate intrinsic value for outstanding options is calculated as the difference between the exercise price of the underlying awards and the quoted price of the Company s common stock as of the last trading day of fiscal 2012. The total intrinsic value of options exercised during the years ended December 31, 2012, 2011 and 2010 was $94.6 million, $25.1 million and $18.0 million, respectively. The total intrinsic value of options exercisable at December 31, 2012 was $226.6 million. The weighted-average remaining contractual lives for options outstanding and options exercisable at December 31, 2012 was 6.9 years and 5.9 years, respectively. There were 13.8 million options that were in-the-money at December 31, 2012. The aggregate intrinsic value of options exercised was determined as of the date of option exercise. Upon the exercise of the options, the Company issues new common stock from its authorized shares.

At December 31, 2012, an aggregate of approximately 12.8 million unissued shares were authorized for future issuance under the Share Incentive Plan.

Determining the Fair Value of Stock Options and Stock Purchase Rights

The fair value of each option award is estimated on the date of grant using the Black-Scholes valuation model and the assumptions noted in the tables below. The expected life of options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes, but none were identified that had distinctly different exercise patterns as of December 31, 2012. The expected volatility of stock options is based upon the weighted average of the historical volatility of the Company s common stock and the implied volatility of traded options on the Company s common stock for fiscal periods in which there is sufficient trading volume in options on the Company s common stock. The risk-free interest rate is based on the implied yield on a U.S. Treasury zero-coupon issue with a remaining term equal to the expected term of the option. The dividend yield reflects that the Company has not paid any cash dividends since inception and does not intend to pay any cash dividends in the foreseeable future. The assumptions used to estimate the per share fair value of stock options granted under the 2012 Inducement Plan and the 2006 Share Incentive Plan were as follows:

	Years Ended December 31,		
	2012	2011	2010
Expected volatility	45-46%	46-50%	50-52%
Dividend yield	0.0%	0.0%	0.0%
Expected life	6.5 years	6.3-6.4 years	6.2 years
Risk-free interest rate	0.8-1.1%	1.2-2.7%	1.8-2.7%

The Company recorded $32.8 million, $31.7 million and $28.7 million of compensation costs related to current period vesting of stock options for the years ended December 31, 2012, 2011 and 2010, respectively. As of December 31, 2012, the total unrecognized compensation cost related to unvested stock options was $70.6 million. These costs are expected to be recognized over a weighted average period of 2.6 years.

The assumptions used to estimate the per share fair value of stock purchase rights granted under the ESPP were as follows:

	Years Ended December 31,		
	2012	2011	2010
Expected volatility	31%	32-48%	50-52%
Dividend yield	0.0%	0.0%	0.0%
Expected life	6-24 months	6-24 months	6-24 months
Risk-free interest rate	0.2-0.3%	0.1-0.6%	0.2-1.0%

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The Company recorded $2.9 million, $2.4 million and $2.4 million of compensation costs related to options granted under the ESPP for the years ended December 31, 2012, 2011 and 2010, respectively. As of December 31, 2012, there was $4.3 million of total unrecognized compensation cost related to unvested stock options issuable under the ESPP. These costs are expected to be recognized over a weighted average period of 1.4 years.

Restricted Stock Units with Service-Based Vesting Conditions

RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

A summary of non-vested restricted stock unit activity under the plan for the year ended December 31, 2012 as follows:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2011	570,904	$ 24.54
Granted	612,270	$ 37.81
Vested	(205,980)	$ 24.85
Forfeited	(78,245)	$ 28.11
Non-vested units as of December 31, 2012	898,949	$ 33.10

The Company recorded $7.3 million, $4.5 million and $2.1 million of compensation costs related to restricted stock units for the years ended December 31, 2012, 2011 and 2010, respectively. As of December 31, 2012, there was $23.9 million of total unrecognized compensation cost related to unvested restricted stock units with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 3.0 years.

Restricted Stock Unit Awards with Performance and Market Vesting Conditions

During 2012 and 2011, pursuant to the Board s approval, the Company granted RSU awards under the Share Incentive Plan and 2012 Inducement Plan to certain executive officers that provide for a base award of 875,000 RSUs in total (Base RSUs) that may be adjusted to 75% to 125% depending on the performance of the Company s stock as discussed further below. A summary of non-vested Base RSU activity under the plans for the year ended December 31, 2012 is as follows:

	Base Awards	Weighted Average Grant Date Fair Value
Non-vested units with performance and market vesting conditions as of December 31, 2011	875,000	$ 32.61

Granted	140,000	$ 40.26
Vested	0	
Forfeited	(140,000)	$ 32.61
Non-vested units with performance and market vesting conditions as of December 31, 2012	875,000	$ 33.83

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The vesting of the Base RSUs under these specific grants is contingent upon the achievement of multiple performance conditions, as follows:

Strategic Performance Goals	Percentage of Base RSUs to Vest Upon Achievement of Goal	Base Number of RSUs Granted Before TSR Multiplier
Product Goals		
Approval of Vimizim in the U.S. or EU prior to December 31, 2015	35%	306,250
Approval of PEG-PAL or any other non-Vimizim product in the U.S. or EU prior to December 31, 2015	25%	218,750
Financial Goal		
Total revenues of at least $775.0 million in fiscal 2015	40%	350,000
		875,000

The number of RSUs that could potentially vest from the Base RSUs granted is contingent upon achievement of specific performance goals and will be multiplied by the Total Shareholder Return (TSR) multiplier which could range from 75% to 125% to determine the number of earned RSUs. The TSR multiplier will be determined based on the Company s TSR percentile ranking relative to the TSR of the NASDAQ Biotechnology Index on December 31, 2015. TSR is calculated based on the 20-trading day average prices before the beginning and end of the performance period of the Company s common stock and each comparator company in the NASDAQ Biotechnology Index. The measurement period for the performance and TSR conditions is from the grant date through December 31, 2015, subject to certain change of control provisions (the Performance Period). The Company s TSR percentile ranking within the NASDAQ Biotechnology Index will result in a TSR multiplier ranging from 75% to 125%. The RSUs earned at the end of the Performance Period will vest on the filing date of the Company s Annual Report on Form 10-K for the 2015 fiscal year, subject to certain holding periods. The maximum number of RSUs that could vest if all performance conditions are achieved and a TSR multiplier of 125% is applied would be 1,093,750 RSUs.

The Company utilized a Monte Carlo simulation model to estimate the TSR multiplier and determined the grant date fair value on each of the grant dates. The assumptions used to estimate the fair value of the RSUs with performance and market vesting conditions were as follows:

	Grant Date		
	September 5, 2012	May 29, 2012	June 1, 2011
Fair value of the Company s common stock on grant date	$ 37.45	$ 39.06	$ 28.11
Expected volatility	31.73%	44.87%	47.95%
Risk-free interest rate	0.37%	0.52%	1.42%
Dividend yield	0.0%	0.0%	0.0%

The Monte Carlo simulation model also assumed correlations of returns of the stock prices of the Company s common stock and the common stock of a peer group of companies and historical stock price volatilities of the peer group of companies. The valuation model also used terms based on the length of the performance period and compound annual growth rate goals for total stockholder return based on the provisions of the award.

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Stock-based compensation expense for this award will be recognized over the remaining service period beginning in the period the Company determines the strategic performance goal or goals is probable of achievement. Accordingly, because the Company s management has not yet determined the goals are probable of achievement as of December 31, 2012, no compensation expense has been recognized for these awards for the years ended December 31, 2012 and 2011.

Compensation expense included in the Company s Consolidated Statements of Operations for all stock-based compensation arrangements was as follows:

	Years Ended December 31,		
	2012	2011	2010
Cost of sales	$ 4,890	$ 5,171	$ 4,269
Research and development	20,736	16,365	13,760
Selling, general and administrative	22,346	22,283	19,463
Total stock-based compensation expense	$ 47,972	$ 43,819	$ 37,492

Stock-based compensation of $4.3 million, $5.3 million and $5.1 million was capitalized into inventory, for the years ended December 31, 2012, 2011 and 2010, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

At December 31, 2012, an aggregate of approximately 15.1 million unissued shares was authorized for future issuance under the Company s stock plans, which includes shares issuable under the Share Incentive Plan, the 2012 Inducement Plan and the Company s ESPP. Under the Share Incentive Plan and the 2012 Inducement Plan, awards that expire or are cancelled without delivery of shares generally become available for issuance under the respective plan. Awards that expire or are cancelled under the Company s suspended 1997 Stock Plan or 1998 Director Option Plan may not be reissued.

(15) EARNINGS (LOSS) PER SHARE

Potential shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the ESPP, unvested restricted stock, common stock held by the Company s Nonqualified Deferred Compensation Plan and contingent issuances of common stock related to convertible debt.

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The following table sets forth the computation of basic and diluted earnings/loss per common share:

	Years Ended December 31,		
	2012	2011	2010
Numerator:			
Net income (loss), basic	$ (114,347)	$ (53,836)	$ 205,819
Interest expense on convertible debt	0	0	9,977
Amortization of deferred offering costs related to the convertible debt	0	0	1,549
Net income (loss), diluted	$ (114,347)	$ (53,836)	$ 217,345
Denominator (in thousands of common shares):			
Basic weighted-average shares outstanding	120,271	112,122	103,093
Effect of dilutive securities:			
Stock options	0	0	2,403
Potentially issuable restricted common stock	0	0	286
Potentially issuable common stock for ESPP purchases	0	0	763
Common stock issuable under convertible debt	0	0	19,129
Fully diluted weighted-average shares	120,271	112,122	125,674
Basic earnings (loss) per common share	$ (0.95)	$ (0.48)	$ 2.00
Diluted earnings (loss) per common share	$ (0.95)	$ (0.48)	$ 1.73

In addition to the equity instruments included in the table above, the table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method:

	Years Ended December 31,		
	2012	2011	2010
Options to purchase common stock	13,895	16,319	12,497
Common stock issuable under convertible debt	17,365	17,372	0
Unvested restricted stock units	1,165	1,068	134
Potentially issuable common stock for ESPP purchases	263	241	0
Common stock held by the Nonqualified Deferred Compensation Plan	233	173	104
Total	32,921	35,173	12,735

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(16) ACQUISITION OF ZYSTOR THERAPEUTICS, INC.

On August 17, 2010, the Company acquired all of the capital stock of ZyStor Therapeutics, Inc. (ZyStor), a privately held biotechnology company, pursuant to a securities purchase agreement dated August 17, 2010 between the Company, ZyStor, the holders of outstanding capital stock and rights to acquire capital stock of ZyStor and the representative of such holders. ZyStor engaged in developing enzyme replacement therapies for the treatment of lysosomal storage disorders. ZyStor s lead product candidate, ZC-701, now referred to as BMN-701, is a novel fusion of insulin-like growth factor 2 and alpha glucosidase in development for the treatment of Pompe disease.

In connection with its acquisition of ZyStor, the Company paid $20.3 million, net of transaction costs, upfront for all of the outstanding common stock of ZyStor. Additionally at the closing, the Company held back $2.0 million of the purchase price as indemnification against possible claims to pay any unidentified obligations of the former ZyStor stockholders. The Company also agreed to pay the ZyStor stockholders additional consideration in future periods of up to $93.0 million (undiscounted) in milestone payments if certain annual sales, cumulative sales and development milestones are met. The fair value of the contingent acquisition consideration payments on the acquisition date was $15.6 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as level 3 inputs. Key assumptions included a discount rate of 5.6% and various probability factors. The range of outcomes and assumptions used to develop these estimates have been updated to estimate the fair value of the contingent acquisition consideration payable at December 31, 2012 (see Note 12 for additional discussion regarding fair value measurements of the contingent acquisition consideration payable).

The following table presents the allocation of the purchase consideration, including the contingent acquisition consideration payable, based on fair value:

Upfront cash payments	$ 20,250
Present value of cash held back at closing	1,890
Contingent acquisition consideration payable	15,560
Transaction costs included in Selling, General & Administrative (SG&A) expense	(1,751)
Total consideration	$ 35,949
Cash and cash equivalents	$ 13
Other current assets	14
Property, plant and equipment	54
Acquired deferred tax assets	7,600
Intangible assets In Process Research & Development (IPR&D)	27,600
Total identifiable assets acquired	$ 35,281
Accounts payable and accrued expenses	$ (1,644)
Deferred tax liability	(10,692)
Total liabilities assumed	$ (12,336)
Net identifiable assets acquired	$ 22,945
Goodwill	13,004
Net assets acquired	$ 35,949

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A substantial portion of the assets acquired consisted of intangible assets related to ZyStor s lead product candidate, which the Company refers to as BMN-701. The Company determined that the estimated acquisition-date fair values of the intangible assets related to the lead product candidate were $25.0 million. See Note 6 for further discussion related to intangible assets.

The $7.6 million of deferred tax assets resulting from the acquisition was primarily related to federal and state net operating loss and tax credit carryforwards. The $10.7 million of deferred tax liabilities relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes.

The excess of the consideration transferred over the fair values assigned to the assets acquired and liabilities assumed was $2.3 million, which represents the amount of goodwill resulting from the acquisition. The Company believes that the goodwill primarily represents the synergies and economies of scale expected from combining the Company s operations with those of ZyStor. None of the goodwill is expected to be deductible for income tax purposes. The Company recorded the goodwill in the Company s consolidated balance sheet as of the acquisition date.

The Company recognized $1.8 million of acquisition-related transaction costs in selling, general and administrative expenses during 2010, which consisted primarily of investment banker fees, legal fees and transaction bonuses to former ZyStor employees and directors related to the acquisition.

(17) ACQUISITION OF LEAD THERAPEUTICS, INC.

On February 10, 2010, the Company acquired LEAD Therapeutics, Inc. (LEAD), a small private drug discovery and early stage development company with a key compound which the Company refers to as BMN-673, an orally available poly (ADP-ribose) polymerase (PARP) inhibitor for the treatment of patients with certain cancers for a total purchase price of $39.1 million.

In connection with its acquisition of LEAD, the Company paid $18.6 million in cash upfront for all of the outstanding common stock of LEAD. The Company also agreed to pay the LEAD stockholders additional consideration in future periods of up to $68.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met. The fair value of the contingent acquisition consideration payments was $20.5 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as level 3 inputs. Key assumptions included a discount rate of 6.4% and various probability factors. The range of outcomes and assumptions used to develop these estimates have been updated to estimate the fair value of the contingent consideration payable as of December 31, 2012 (see Note 12 for additional discussion regarding fair value measurements of the contingent acquisition consideration payable). In December 2010, the Medicines and Healthcare Products Regulatory Agency in the United Kingdom completed its review of the Company s Clinical Trial Application and issued a notice of acceptance for BMN-673 resulting in a payment of a regulatory milestone of $11.0 million to the former LEAD stockholders. In October 2012, the Company paid the former LEAD stockholders $6.0 million for the attainment of a clinical milestone.

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The following table presents the allocation of the purchase consideration, including the contingent acquisition consideration payable, based on fair value:

Cash and cash equivalents	$ 1,187
Prepaid expenses	40
Property, plant and equipment	26
Acquired deferred tax assets	7,788
Intangible assets IPR&D	36,089
Total identifiable assets acquired	$ 45,130
Accounts payable and accrued expenses	$ (891)
Deferred tax liability	(13,981)
Valuation allowance for acquired deferred tax assets	(7,788)
Total liabilities assumed	$ (22,660)
Net identifiable assets acquired	$ 22,470
Goodwill	16,638
Net assets acquired	$ 39,108

The deferred tax liability relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes. The $16.6 million of goodwill reflects the $14.0 million deferred tax liability recognized in connection with the LEAD acquisition and $2.6 million of goodwill attributable to the synergies expected from the acquisition and other benefits that do not qualify for separate recognition as acquired intangible assets.

See Note 6 for further discussion of the acquired intangible assets.

(18) REVENUE AND CREDIT CONCENTRATIONS

Net Product Revenue The Company considers there to be revenue concentration risks for regions where net product revenue exceeds ten percent of consolidated net product revenue. The concentration of the Company s net product revenue within the regions below may have a material adverse effect on the Company s revenue and results of operations if sales in the respective regions were to experience difficulties.

The table below summarizes net product revenue concentrations based on patient location for Naglazyme, Kuvan and Firdapse and Genzyme s headquarters for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme s net sales are included in the U.S. region, as the transactions are with Genzyme whose headquarters are located in the U.S.

	Years Ended December 31,		
	2012	2011	2010
Region:			
United States	50%	51%	53%
Europe	22%	23%	24%

Latin America	15%	13%	11%
Rest of world	13%	13%	12%
Total net product revenue	100%	100%	100%

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The following table illustrates the percentage of the consolidated net product revenue attributed to the Company s three largest customers.

	Years Ended December 31,		
	2012	2011	2010
Customer A	15%	17%	18%
Customer B (1)	16%	19%	19%
Customer C	12%	10%	9%
Total	43%	46%	46%

(1) Genzyme is the Company s sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Net product revenues from Genzyme are comprised of royalties on worldwide net Aldurazyme sales and incremental product transfer revenue.

The accounts receivable balances at December 31, 2012 and 2011 were comprised of amounts due from customers for net product sales of Naglazyme, Kuvan and Firdapse and Aldurazyme product transfer and royalty revenues. On a consolidated basis, our two largest customers accounted for 51% and 13% of the December 31, 2012 accounts receivable balance, respectively, compared to December 31, 2011 when the two largest customers accounted for 49% and 14% of the accounts receivable balance, respectively. As of December 31, 2012 and 2011, accounts receivable for the Company s largest customer balance included $32.4 million and $31.0 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme. The Company does not require collateral from its customers, but performs periodic credit evaluations of its customers financial condition and requires immediate payment in certain circumstances.

The Company s product sales to government-owned or government-funded customers in certain European countries, including Italy, Spain, Portugal and Greece are subject to payment terms that are statutorily determined. Because these customers are government-owned or government-funded, the Company may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in these countries may decrease the likelihood that the Company will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to the Company s operating results. For the year ended December 31, 2012, approximately 4% of the Company s net product revenues were from these countries. Additionally, approximately 8% of the Company s outstanding accounts receivable at December 31, 2012 related to such countries.

The following table summarizes the accounts receivable by country that were past due related to Italy, Spain, Portugal and Greece, the number of days past due and the total allowance for doubtful accounts related to each of these countries at December 31, 2012.

	Days Past Due				
	< 180 Days	180 360 Days	> 360 Days	Total Amount Past Due	Allowance for Doubtful Accounts
Italy	$ 0	$ 0	$ 0	$ 0	$ 0
Spain	1,680	510	0	2,190	0
Portugal	0	0	0	0	0
Greece	0	0	348	348	348
Total	$ 1,680	$ 510	$ 348	$ 2,538	$ 348

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The Company has not historically experienced a significant level of uncollected receivables and has received continued payments from its more aged accounts. The Company believes that the allowances for doubtful accounts related to these countries is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

(19) INCOME TAXES

The provision for (benefit from) income taxes is based on income (loss) before income taxes as follows:

	Years Ended December 31,		
	2012	2011	2010
U.S. Source	$ 45,422	$ 63,640	$ 28,659
Non-U.S. Source	(163,700)	(107,267)	(50,149)
Income (loss) before income taxes	$ (118,278)	$ (43,627)	$ (21,490)

The U.S. and foreign components of the provision for (benefit from) income taxes are as follows:

	Years Ended December 31,		
	2012	2011	2010
Provision for current income tax expense (benefit):			
Federal	$ 2,253	$ 2,766	$ 289
State and local	1,879	1,439	1,355
Foreign	1,858	1,641	1,624
	$ 5,990	$ 5,846	$ 3,268
Provision for deferred income tax expense (benefit):			
Federal	$ (6,055)	$ 7,398	$ (213,796)
State and local	(3,891)	(2,957)	(16,377)
Foreign	25	(78)	(404)
	$ (9,921)	$ 4,363	$ (230,577)
Provision for (benefit from) income taxes	$ (3,931)	$ 10,209	$ (227,309)

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The following is a reconciliation of the statutory federal income tax rate to the Company s effective income tax rate expressed as a percentage of income (loss) before income taxes:

	Years Ended December 31,		
	2012	2011	2010
Federal statutory income tax rate	35.0%	35.0%	35.0%
State and local taxes	(1.3)	(1.9)	(6.3)
Orphan Drug & General Business Credit	27.6	43.9	(23.3)
Stock compensation expense	(1.6)	(8.2)	(12.7)
Nondeductible debt conversion expense	0	(0.6)	(10.9)
Changes in the fair value of contingent acquisition consideration payable	(2.6)	1.5	(6.5)
Nondeductible acquisition expenses	0	(0.2)	(1.9)
Section 162(m) limitation	(1.2)	(0.9)	(1.6)
Permanent items	(0.9)	(1.3)	(1.6)
Foreign tax rate differential	(50.0)	(86.7)	(89.2)
Other	(1.1)	1.0	0
Valuation allowance/Deferred benefit	(0.6)	(5.0)	1176.7
Effective income tax rate	3.3%	(23.4)%	1057.7%

The significant components of the Company s net deferred tax assets are as follows:

	December 31,	
	2012	2011
Net deferred tax assets:		
Net operating loss carryforwards	$ 20,431	$ 33,796
Credit and contribution carryforwards	170,322	162,710
Property, plant and equipment	1,791	231
Accrued expenses, reserves, and prepaids	18,770	12,148
Intangible assets	6,161	5,979
Stock-based compensation	22,634	22,144
Inventory	17,074	10,957
Capital loss carryforwards	3,083	3,101
Other	764	167
Gross deferred tax assets	$ 261,030	$ 251,233
Deferred tax liability related to joint venture basis difference	(1,801)	(1,813)
Deferred tax liability related to business acquisitions	(31,420)	(35,127)
Other	(75)	(215)
Valuation allowance	(6,075)	(5,441)
Net deferred tax assets	$ 221,659	$ 208,637

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

As of December 31, 2012, the Company had federal net operating loss carryforwards of approximately $141.5 million and state net operating loss carryforwards of approximately $155.5 million. The Company also had federal research and development and orphan drug credit carryforwards of approximately $188.4 million as of December 31, 2012, and state research credit carryovers of approximately $25.5 million. The federal net operating loss carryforwards will expire at various dates beginning in 2024 through 2031 if not utilized. The federal credit carryforward will expire at various dates beginning in 2018 through 2032 of not utilized. The state net operating loss carryforwards will expire at various dates beginning in 2013 through 2032 if not utilized. Certain state research credit carryovers will begin to expire in 2017 if not utilized, with others carrying forward indefinitely. The Company also has Canadian net operating loss carryforwards of $2.0 million and research credit carryovers of $0.7 million that it currently does not expect to fully utilize and therefore the Company carries a full valuation allowance on all but $0.3 million of the research credit carryforward. The Canadian net operating loss carryforwards and research credit carryovers expire from 2014 to 2027 and from 2018 to 2022, respectively.

The Company has elected to recognize the excess benefits related to the exercise of employee stock options under a with and without approach, which will be accounted for as an increase to additional paid-in-capital if and when realized. As of December 31, 2012, the Company had an unrecognized federal benefit of approximately $139.0 million and an unrecognized state benefit of approximately $50.9 million.

The Company s net operating losses and credits could be subject to annual limitations due to ownership change limitations provided by Internal Revenue Code Section 382 and similar state provisions. An annual limitation could result in the expiration of net operating losses and tax credit carryforward before utilization. There are limitations on the tax attributes of the entities acquired in 2010 however the Company does not believe the limitations will have a material impact on the utilization of the net operating losses or tax credits.

The $31.4 million deferred tax liability relates to the tax impact of future amortization or possible impairments associated with the intangible assets acquired from ZyStor, LEAD and Huxley, which are not deductible for tax purposes.

Based on projected U.S. taxable income and other key operating factors, the Company concluded in 2010 that it is more likely than not that a significant portion of the benefit of its deferred tax assets would be realized. As a result, the amount of the valuation allowance related to the deferred tax assets expected to be realized was reversed, resulting in a net tax benefit in 2010 of $230.6 million, which was recorded as a tax benefit in the Company s consolidated statement of operations in 2010. The financial projections supporting the Company s conclusion to release a portion of its valuation allowance contain significant assumptions and estimates of future operations. If such assumptions were to differ significantly, it may have a material impact of the Company s ability to realize its deferred tax assets. At the end of each period, the Company will reassess the ability to realize the deferred tax benefits. If it is more likely than not that the Company will not realize the deferred tax benefits, then all or a portion of the valuation allowance may need to be re-established, which would result in a charge to tax expense.

In 2012, the valuation allowance increased by $0.6 million due primarily to investment impairments that are not more likely than not to be realized. In 2011 the valuation allowance increased by $1.8 million primarily due primarily to capital losses associated with the investment loss that are not more likely than not to be realized.

Effective January 1, 2007 the Company adopted the accounting requirements that clarified the criteria for recognizing income tax benefits and requires disclosures of uncertain tax positions. The financial statement recognition of the benefit for a tax position is dependent upon the benefit being more likely than not to be

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

sustainable upon audit by the applicable taxing authority. If this threshold is met, the tax benefit is then measured and recognized at the largest amount that is greater than 50% likely of being realized upon ultimate settlement. A reconciliation of the beginning and ending amount of unrecognized tax benefits for the two years ended December 31, 2012 is as follows:

	December 31,	
	2012	2011
Balance at beginning of period	$ 36,350	$ 31,112
Additions based on tax positions related to the current year	7,190	4,660
Additions for tax positions of prior years	(9)	578
Balance at end of period	$ 43,531	$ 36,350

Included in the balance of unrecognized tax benefits at December 31, 2012 are potential benefits of $43.5 million that, if recognized, would affect the effective tax rate. The Company s policy for classifying interest and penalties associated with unrecognized income tax benefits is to include such items in the income tax expense. No interest or penalties have been recorded by the Company to date through December 31, 2012.

The Company files income tax returns in the U.S. federal jurisdiction and various states and foreign jurisdictions. For income tax returns filed before 2010, the Company is no longer subject to audit by the U.S. federal, state, local or non-U.S. tax authorities. However, carryforward tax attributes that were generated prior to 2010 may still be adjusted upon examination by tax authorities. Currently, the Company has no pending or open tax return audits.

U.S. income and foreign withholding taxes have not been recognized on the excess of the amount for financial reporting over the tax basis of investments in foreign subsidiaries that are essentially permanent in duration. This excess totaled approximately $3.9 million as of December 31, 2012, which will be indefinitely reinvested; therefore, deferred income taxes of approximately $1.4 million have not been provided on such foreign earnings.

(20) COLLABORATIVE AGREEMENTS

Merck Serono

In May 2005, the Company entered into an agreement with Merck Serono S.A. (Merck Serono) for the further development and commercialization of BH4, both in Kuvan for PKU and for other indications, and PEG-PAL (phenylalanine ammonia lyase). Through the agreement and subsequent amendment, Merck Serono acquired exclusive rights to market these products in all territories outside the U.S., Canada and Japan, and BioMarin retained exclusive rights to market these products in the U.S. and Canada. The Company and Merck Serono may collaborate on the development of Kuvan and PEG-PAL. If they agree to collaborate Merck Serono will generally share equally all development costs following successful completion of Phase 2 trials for such product candidate in such indication. Merck Serono has opted-out of the PEG-PAL development program, a decision that does not affect its exclusive rights to PEG-PAL in its territory. Unless or until Merck Serono elects to opt-in, it is not obligated to pay any of the milestones related to the program or to reimburse the Company for any of the PEG-PAL development costs. Merck Serono may elect to opt in at any time. If it elects to opt in prior to the unblinding of the first Phase 3 trial, it must pay 75% of the Phase 3 costs incurred prior to opting in and a $7.0 million development milestone if the Phase 3 trial has started. If Merck Serono opts in after the unblinding of the first Phase 3 trial for PEG-PAL, it must pay 100% of the Phase 3 costs incurred prior to opting in and a $7.0 million development milestone.

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

BioMarin and Merck Serono are individually responsible for the costs of commercializing the products within their respective territories. Merck Serono will also pay BioMarin royalties on its net sales of these products. The term of the agreement is the later of 10 years after the first commercial sale of the products or the period through the expiration of all related patents within the territories. As of December 31, 2012 and 2011, amounts due from Merck Serono for reimbursable development costs for Kuvan totaled $0.4 million and $0.1 million, respectively.

Other Agreements

The Company is engaged in research and development collaborations with various other entities. These provide for sponsorship of research and development by the Company and may also provide for exclusive royalty-bearing intellectual property licenses or rights of first negotiation regarding licenses to intellectual property development under the collaborations. Typically, these agreements can be terminated for cause by either party upon 90 days written notice.

In September 2007, the Company licensed to Asubio Pharma Co., Ltd. (a subsidiary of Daiichi Sankyo) exclusive rights to data and intellectual property contained in the Kuvan new drug application. The Company receives royalties on net sales of the product in Japan.

In October 2012, the Company licensed to Catalyst Pharmaceutical Partners, Inc., the North American rights to develop and market Firdapse. In consideration of this licensing arrangement, the Company received from Catalyst a $5.0 million convertible promissory note. Under the terms of the note agreement, the Company received 6.7 million shares of Catalyst common stock upon the automatic conversion of the convertible promissory note on December 10, 2012. The conversion price was based on $0.75 per share, which resulted in a $2.0 million loss on conversion, which was included as a component of Other Income (Expense) on the Company s Consolidated Statement of Operations for the year ended December 31, 2012. In exchange for the North American rights to Firdapse the Company may receive royalties of 7% to 10% on net product sales of Firdapse in North America.

(21) COMPENSATION AGREEMENTS AND PLANS

Employment Agreements

The Company has entered into employment agreements with certain officers. Generally, these agreements can be terminated without cause by the Company upon prior written notice and payment of specified severance, or by the officer upon four weeks prior written notice to the Company.

401(k) Plan

The Company sponsors the BioMarin Retirement Savings Plan (401(k) Plan). Most employees (Participants) are eligible to participate following the start of their employment, at the beginning of each calendar month. Participants may contribute to the 401(k) Plan up to the lesser of 100% of their current compensation to or an amount up to a statutorily prescribed annual limit. The Company pays the direct expenses of the 401(k) Plan and matches 100% of each Participant s contributions, up to a maximum of the lesser of 2% of the employee s annual compensation or $4,000 per year. The Company s matching contribution vests over four years from employment commencement and was approximately $2.8 million, $2.2 million and $1.4 million for the years ended December 31, 2012, 2011 and 2010, respectively. Employer contributions not vested upon employee termination are forfeited.

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Deferred Compensation Plan

In December 2005, the Company adopted the Deferred Compensation Plan. The Deferred Compensation Plan allows eligible employees, including members of the Board, management and certain highly-compensated employees as designated by the Plan s Administrative Committee, the opportunity to make voluntary deferrals of compensation to specified future dates, retirement or death. Participants are permitted to defer portions of their salary, annual cash bonus and restricted stock. The Company may not make additional direct contributions to the Deferred Compensation Plan on behalf of the participants, without further action by the Board. Deferred compensation is held in trust and generally invested to match the investment benchmarks selected by participants. The recorded cost of any investments will approximate fair value. Investments of $4.4 million and $3.5 million and the related deferred compensation liability of $15.9 million and $9.5 million were recorded as of December 31, 2012 and 2011, respectively. Company stock issued into the Deferred Compensation Plan is recorded and accounted for similarly to treasury stock in that the value of the employer stock is determined on the date the restricted stock vests and the shares are issued into the Deferred Compensation Plan. The restricted stock issued into the Deferred Compensation Plan upon vesting is recorded in stockholders equity. As of December 31, 2012 and 2011, the fair value of Company stock held by the Deferred Compensation Plan was $11.5 million and $5.9 million, respectively. The change in market value amounted to a loss of approximately $3.2 million in 2012, compared to losses of $1.3 million in 2011 and $0.8 million in 2010.

(22) JOINT VENTURE

Effective January 2008, the Company and Genzyme restructured BioMarin/Genzyme LLC. Under the revised structure, the operational responsibilities for the Company and Genzyme did not significantly change, as Genzyme continues to globally market and sell Aldurazyme and the Company continues to manufacture Aldurazyme.

Genzyme records sales of Aldurazyme to third-party customers and pays the Company a tiered payment ranging from approximately 39.5% to 50% of worldwide net product sales depending on sales volume, which is recorded by the Company as product revenue. The Company recognizes a portion of this amount as product transfer revenue when the product is released to Genzyme because all of the Company s performance obligations are fulfilled at this point and title to, and risk of loss for, the product has transferred to Genzyme. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay the Company if the product is unsold by Genzyme. The amount of product transfer revenue is deducted from the calculated royalty rate when the product is sold by Genzyme. Genzyme s contractual return rights for Aldurazyme are limited to defective product. Certain research and development activities and intellectual property related to Aldurazyme continue to be managed in the joint venture with the costs shared equally by the Company and Genzyme.

The Company presents the related cost of sales and its Aldurazyme-related operating expenses as operating expenses in the Consolidated Statements of Operations. Equity in the loss of BioMarin/Genzyme LLC subsequent to the restructuring includes BioMarin s 50% share of the net income (loss) of BioMarin/Genzyme LLC related to intellectual property management and ongoing research and development activities.

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The results of the joint venture s operations are presented in the table below.

	Years Ended December 31,		
	2012 (unaudited)	2011 (unaudited)	2010 (unaudited)
Revenue	$ 0	$ 0	$ 0
Cost of goods sold	0	0	0
Gross profit	0	0	0
Operating expenses	2,534	4,855	5,938
Loss from operations	(2,534)	(4,855)	(5,938)
Other income (expense)	4	5	(43)
Net loss	$ (2,530)	$ (4,850)	$ (5,981)
Equity in the loss of BioMarin/Genzyme LLC	$ (1,221)	$ (2,426)	$ (2,991)

The summarized assets and liabilities of the joint venture and the components of the Company s investment in the joint venture are as follows:

	December 31,	
	2012 (unaudited)	2011 (unaudited)
Assets	$ 3,343	$ 2,531
Liabilities	(1,747)	(1,406)
Net equity	$ 1,596	$ 1,125
Investment in BioMarin/Genzyme LLC (50% share of net equity)	$ 1,080	$ 559

(23) COMMITMENTS AND CONTINGENCIES

Lease Commitments

The Company leases office space and research, testing and manufacturing laboratory space in various facilities under operating agreements expiring at various dates through 2022. Certain of the leases provide for options by the Company to extend the lease for multiple five-year renewal periods and also provide for annual minimum increases in rent, usually based on a consumer price index or annual minimum increases. Minimum lease payments for future years are as follows:

2013	$ 9,015
2014	6,749

2015	6,990
2016	6,496
2017	5,780
Thereafter	22,226
Total	$ 57,256

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Rent expense for the years ended December 31, 2012, 2011 and 2010 was $10.1 million, $6.0 million, and $5.1 million, respectively. Deferred rent accruals at December 31, 2012 totaled $10.0 million, of which $1.0 million was current. At December 31, 2011 deferred rent accruals totaled $1.3 million, of which $0.3 million was current.

Research and Development Funding and Technology Licenses

The Company uses experts and laboratories at universities and other institutions to perform certain research and development activities. These amounts are included as research and development expenses as services are provided.

The Company has also licensed technology, for which it is required to pay royalties upon future sales, subject to certain annual minimums. As of December 31, 2012, such minimum annual commitments were approximately $0.6 million.

Contingencies

From time to time the Company is involved in legal actions arising in the normal course of its business. The Company is not presently subject to any material litigation nor, to management s knowledge, is any litigation threatened against the Company that collectively is expected to have a material adverse effect on the Company s consolidated cash flows, financial condition or results of operations.

As of December 31, 2012 the Company is also subject to contingent payments totaling approximately $285.5 million upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. As of December 31, 2012, the Company has recorded $41.4 million of contingent acquisition consideration payable on its Consolidated Balance Sheet, of which $10.8 million current.

(24) SUBSEQUENT EVENT

On January 4, 2013, the Company entered into a merger agreement with Zacharon Pharmaceuticals, Inc. (Zacharon), a private biotechnology company focused on developing small molecules targeting pathways of glycan and glycolipid metabolism, pursuant to which the Company acquired all of the outstanding shares of capital stock of Zacharon for an upfront cash payment to the stockholders of Zacharon of $10.0 million, of which $1.7 million was held in escrow. Additionally, the Company may pay the former Zacharon stockholders up to $134.0 million for the achievement of specified development and launch milestones.